



## PMPRB Guideline Submission February 14th, 2020

Thank you for the opportunity to provide feedback on the draft Guidelines published in late November 2019.

We, the signatories to this submission, will provide below some background to our deliberations to set the context through which we considered the Guidelines, provide case studies for your review (attached), provide comments about technical areas of concern about the Guidelines, describe substantive issues from an oncology specific perspective with the Guidelines and provide recommendations to remedy those concerns.

### Background

We are aware of the challenges of a dual federal/provincial jurisdiction for aspects of healthcare for people across Canada.

We are also aware that there are inequities in coverage for medications across the country due to several factors. Public systems are the responsibility of provinces and territories and of course each has its own economic engine, priorities, demographics and other factors that drive decisions about how much to spend, what to fund and what funding models to implement. In addition, employers, unions and individuals who can afford them have private plans that provide additional access.

Patients understand that this is the construct we have. We understand that public plans cannot afford to provide access to all drugs that we might need although we trust that they will use instruments that will help make fair, objective and evidence-based choices. We want a sustainable system; we want the prices of drugs to permit sustainability. This is no doubt the appeal of a universal single payer pharmacare plan.

Patient groups continue to support health technology assessment agencies, CADTH and INESSS, and also pCPA. As for PMPRB, over 20 years ago, the Canadian Treatment Action Council, a

national patient driven HIV organization, chaired by Louise Binder, a representative of one of the signatories to this submission, advocated strongly to the PMPRB and the then federal Minister of Health, (including holding a public protest) that the Regulations for PMPRB be amended to remove the U.S. from the basket as an outlier, with high drug prices, and with health policies inconsistent with our health system. Finally, this is happening.

Patient organizations support the reassessment of the current basket of reference countries proposed for PMPRB consideration.

It is also important in our view to remember the history of cancer management in Canada:

- In 2007 the House of Commons Standing Committee on Health heard evidence that cancer treatments required their own health technology assessment process to ensure that value is analyzed based on factors relevant to that complex group of diseases. The Committee agreed with these recommendations. The result of those hearings was the creation of pCODR with a four-part deliberative Framework as its HTA process.
- The federal government also created a cancer strategy stewarded by the Canadian Partnership Against Cancer.
- Provinces have cancer agencies to manage cancer generally, including drug reimbursement, separately from other treatments.

The statistics regarding cancer certainly make the case for this specific focus on cancer:

- It is the number 1 cause of death in Canada.
- It is estimated that 1 of 2 people in Canada will be diagnosed with cancer in their lifetime.
- 1 in 4 will die from it, or 821,000 in Canada.

This is a huge public health issue that requires a discreet policy approach, as the governments have recognized.

In addition, as we know, there is not just one type of cancer, one stage of cancer or one cause of cancer:

- There are cancers that are uncommon and those that are more common.
- There are cancers for which research has found genetic links that inform prevention and treatment and those that have not.
- There are cancers that can be cured.
- There are cancers that can be effectively and have been transformed into chronic illnesses with newer, more effective treatments; yet, there are many that continue to be a certain death sentence within months of diagnosis.

Breast cancer: The most common cancer among women, both young and older people alike, is breast cancer. In 2019, 26,900 women were diagnosed with breast cancer and 5,000 will die of this disease. 230 men will also be diagnosed with breast cancer. Among women, between 5 and 10% of breast cancers are thought to be hereditary. The BRCA1 and BRCA2 have been known to be linked with a higher risk of breast cancer. More recently, a study in 2017 found 72 new genetic mutations linked to breast cancer and now under study.

Colorectal cancer: Colorectal cancer is the number 2 leading cause of death from a cancer. 50% of cases are diagnosed at stage III and IV and 9600 died of this cancer in 2019.

Lung cancer: Lung cancer has a 29,300 incidence and 21,000 will die each year in Canada. It is the leading cause of cancer deaths for males and females at 25 % and 26 % respectively. Contrary to public opinion, it is not just a disease of those who smoke. Unlike breast cancer, no genetic links have been discovered related this disease. There are certain “signatures” within the lung cancer cell that determine which oral cancer therapies to use. It has a 19% five year survival rate compared to 93 % in prostate cancer, 88% in breast and 65% in colorectal.

Uncommon cancers: There are also uncommon cancers like gastrointestinal stromal tumours (GIST) with unknown prevalence and incidence levels in Canada but estimated at about 400 cases. Genetic testing is recommended to guide treatment decisions for high risk resected and advanced GIST

Paediatric cancers: In young people, approximately 3,800 children, adolescents and young adults aged 0 to 29 years of age are diagnosed with cancer each year in Canada in 2019. 1,500 children and adolescents aged between 0 and 19 were diagnosed with cancer in Canada. Cancer is the second most common cause of death for children in the developed world, after accidents. 416 Canadian children will die of cancer every year. The five-year survival rate for Canadian children has improved from 71% to 82% due to access to new treatments. Without treatment these cancers are fatal.

In our submission the PMPRB, as another agency of the federal government, must recognize that government policy has determined that oncology is a discreet group of diseases for public policy purposes. The signatories to this submission strongly support public policy in this regard.

Until the Guidelines were issued in late November 2019, patient groups had no defined concrete formula or processes by which to determine whether the planned changes will consider public policy regarding oncology and, therefore, whether it will be a good public policy instrument. We do not want another health technology instrument that is less robust than those we presently have.

In order to ensure an objective, evidence based and expert analysis of the Guidelines, we asked an external consultant to assist us. Specifically we asked him to look at a number of oncology drugs for different types of cancer that have been reviewed by pCODR fairly recently and to compare the outcomes they received through that process with the outcomes we can predict they would have had under the proposed Guidelines with the information available to us.

### Case studies

Attached are the six case studies analyzed by the health economics expert. We chose these because they are drugs that have been reviewed recently by pCODR so we have numbers for them – at least those that are in the public domain.

### Conclusions

It is clear that oncology needs its own approach in these Guidelines, as the federal and provincial governments have recognized in other health policies. It is clear that this approach must be flexible enough to recognize the differences between uncommon and more common cancers, difference stages of cancer, genetic factors, paediatrics versus adults, comorbidities, Indigenous populations and social determinants of health, to name a few. This blunt instrument may be workable for some diseases. The case study analysis clearly demonstrates that we need a much more nuanced, flexible and pragmatic instrument is required for cancers.

In two cases the impact of the Guidelines is minimal in terms of pricing changes required and probably will not change the decision about whether or when the drug will come to market in Canada.

In the vast majority of cases, however, the use of this one blunt health technology instrument, the single criterion of an ICER, will generally not make it a practical economic business decision to bring this drug to Canada, or at least not to put it high on the list for applications relative to other countries for market entry.

CADTH's deliberative framework for oncology drugs, with four considerations, including clinical benefit, cost effectiveness, patient values and feasibility of adoption, has recognized this nuanced, flexible and pragmatic health technology assessment required for oncology drugs. This is a recognition that there are limitations of using a single outcome measure for economic evaluation, since doing so that important health consequences are excluded. INESSS also takes into account public and patient values into its health technology assessment considerations.

### Recommendation #1

The Guidelines be amended to adopt explicitly the CADTH pCODR deliberative framework for oncology health technology assessment and remove any specific reference to an ICER.

#### Recommendation #2

The Guidelines be amended to provide that PMPRB's public decision will provide information that the analysis has either met the PMPRB threshold and is not excessive or that it has not met the PMPRB threshold or other CADTH analysis and is excessive. No specific economic data or numbers supporting this decision will be made public by PMPRB. This will ensure that the public Canadian price will not put at risk the U.S. market such that companies will decline to enter, or delay, the Canadian market for that reason.

#### Recommendation #3

The Guidelines be amended to provide for ongoing monitoring and evaluation by a multi-stakeholder Committee and a publicly issued annual report of findings of this Committee. At least two patient group representatives chosen by the patient community, with one from the cancer community, will be included.

#### Recommendation #4

No Guideline finalization should take place until a full consultation is undertaken with Quebec stakeholders including patient groups and patients with all documents and consultations taking place in both Official languages.

#### Recommendation #5

No Guideline finalization should take place until a full consultation is undertaken with Indigenous stakeholders *i.e.* First Nations, Metis and Inuit including patient groups and patients following a process of their choosing.

#### Technical issues to be resolved

1. PMPRB states that it will rely on the base case reanalysis conducted by the public agency (i.e. CADTH and/or INESSS). pCODR does not presently generally do a base case reanalysis and INESSS does one in some cases but not all. This will require coordination amongst the agencies.

2. pCODR builds in the price for companion diagnostics. The manner in which this will be analyzed and taken into account by PMPRB must be clarified and should be described in the Guidelines.
3. pCODR does not do weighted averages for subgroups. PMPRB requires these. This will require coordination between the agencies for resolution.

Martine Elias, Chair  
CONNECTed

Kathleen Barnard, President and Founder  
Save Your Skin Foundation

Elizabeth Lye, Director of Research & Programs  
Lymphoma Canada

Martine Elias, Executive Director:: Directrice Générale  
Myeloma Canada

Christina Sit, Patient Advocate  
Lung Cancer Canada