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Re: PMPRB Draft Guidelines Consultation – Revised Guidelines of June 2020

The following comments are provided by Canadian PKU and Allied Disorders Inc. (CanPKU), a patient advocacy and support non-profit, in response to the Patented Medicine Prices Review Board Revised Draft Guidelines Consultation, as published June 19, 2020. The evidence we hereby submit reiterates the need for sober second thought before the draft guidelines and indeed the new Regulations take effect on January 1, 2021.

CanPKU was a signatory to the Best Medicines Coalition (BMC) comments filed in response to the proposed regulatory changes and the proposed Guidelines. BMC called for a balanced and fair regulatory framework for pharmaceutical pricing aimed at sustaining the life, health and wellbeing of patients. BMC described the goal of a regime that that facilitates the introduction and availability of a comprehensive range of medicines, with the ability for patients to access necessary medicines in a timely manner.

CanPKU filed comments in response to the draft Guidelines, supporting the BMC position and highlighting the fact that the draft Guidelines do not seem designed to prevent excessive pricing – but rather to manage the expenditures of public drug plans or to establish a “reasonable” or “affordable” price. Further, CanPKU raised a number of issues that have not been addressed, either in the revised Guidelines, or in the “Backgrounder” document. Specifically, CanPKU called on the PMPRB to address the following:
• The absence of a gender-based analysis, as required by federal policy, to ensure the Guidelines and Regulations do not have a disproportionate negative impact on women and mothers;
• The duplicative application of price constraints through the new list of reference countries plus the additional economic factors;
• The absence of a true risk-based approach to the guidelines and Regulations; and
• The inappropriate application of the “perspective” of the public health system for the Guidelines.

None of these issues have been acknowledged or addressed, and as a result, the problems they pose remain in the revised Guidelines. Nothing in the revised Guidelines changes the underlying problems with the Regulations, or the problems with the interpretation and application of the Regulations by PMPRB staff.

Under the revised Guidelines, the ultimate maximum regulated price for any patented medicine in Canada is effectively the OECD median, and everything else in the Guidelines pushes the “non-excessive” price below that. CanPKU provided substantive comments on the specific application of the new “factors” in our previous comments. The June revisions have made changes at the margin, but retain the intent and outcomes of the original Guidelines. As a result, CanPKU will not provide specific comments on the tweaks to the individual new factors. Our previous comments remain valid.

Balance of Interests?

CanPKU shares the BMC position that that Canada needs effective, balanced, and fair pharmaceutical pricing rules which contribute to sustaining and improving the health and wellbeing of current and future patients. Regulations and guidelines must achieve the following:

**Improved Affordability of Medicines.** We support the goal of improving the affordability of medicines, both for individual patients, health care systems and private and public insurance. Patients and their families, and those who pay on their behalf, bear a significant burden of prescription drug costs, and we support the government’s intention to address this, particularly in relation to appropriate international comparators.

**Comprehensive Access to Medicines.** Of equal importance, patients need timely access to new drugs which meet unmet needs. There must be confidence, based on best available evidence, that policies, regulations and guidelines will facilitate and not discourage rapid introduction of a full range of medicines and vaccines and access to clinical trials sponsored by drug developers which provide willing patients early access to promising new therapies.

**Accountable, Transparent and Inclusive Governance.** Canadians expect that public health care agencies adopt up-to-date governance which upholds and demonstrates transparency and accountability. Relevant stakeholders, including patients, must be included in policy setting and decision making. PMPRB has work to do to begin to approach best practices in this regard.
CanPKU continues to call for a Canadian drug pricing regime that facilitates the rapid introduction and availability of a comprehensive range of medicines and that provides Canadians the ability to access necessary medicines in a timely manner. The Canadian public health care system, unlike private insurance, has demonstrated its ability to avoid an appropriate solution for patient access more than 10 years for those PKU patients who need the clinical benefits of the first drug to treat PKU. It beggars belief that - under the proposed Guidelines as falsely claimed by some in government - Canada will continue to represent a market that that facilitates the introduction and availability of a comprehensive range of medicines. CanPKU submits that the evidence of misperformance and indeed misconduct of government drug programs and their officials regarding the drug Kuvan over a period of ten years is a very bad sign for what will happen to other newer treatments for PKU approved and funded elsewhere or under development – such as curative cell and gene therapies. The evidence of the Kuvan saga among the government drug programs strongly indicates that these new therapies will not be available in Canada in a timely fashion, if ever.

It is hard to reconcile the idea of Canada being a market that facilitates the introduction and availability in a timely fashion of a comprehensive range of medicines - including new treatments – when a manufacturer cannot seek a list price above the middle of price levels in OECD countries. We believe the “backgrounder” document is the first time that anyone in the government of Canada has explicitly stated this as a policy goal. Here is that statement:

*As a general rule, the Board feels that Canadian list prices higher than international norms smacks of excessiveness and the MIP is an appropriate litmus test for ensuring that Canadian list prices do not become excessive in the future*¹

This objective has been implicit throughout the process since the PMPRB *Guidelines Modernization* discussion paper. The problem is that government officials have been fastidious about avoiding any frank and clear statements of intent or goals for the regulatory changes. It is difficult to have a transparent and fruitful public discussion when the vision for changes are withheld from public understanding or scrutiny. In any event, it is clear that this goal could and would be accomplished simply by using price referencing with the new list of countries.

*CanPKU calls on the PMPRB and the Government of Canada to make public and explicit what are the goal(s) for drug price reductions.*

**Evidentiary Basis for Policy Decisions**

On the topic of transparency and openness in the development of the Regulations and Guidelines, we note that unlike any other Canadian regulator, PMPRB staff produce, develop and editorialize the data and statistics underpinning policy decisions. Policy making and data collection/reporting are normal and legitimate activities of a regulator. Canadians should expect that those activities are separated in a way that ensures data informs decision making, rather than decisions informing data selection. Unusually, there is a palpable linkage between PMPRB policy development and the way that data is collected and presented.

¹ PMPRB Backgrounder on June 2020 Guidelines, p.7
We will highlight the fact that PMPRB held 3 “research” webinars during the consultation period for revised Guidelines. These webinars covered 6 different topics about “analyses expected to inform the consultation”. PMPRB staff presented data in 89 mostly new charts. The last webinar was scheduled 4 days before the original comment deadline. The timing for these webinars and the data presented was not conducive to meaningful review and comment. Frankly, the content and conclusions of the presentations were clearly prepared to support and reinforce existing PMPRB staff positions and proposals. As a participant in the Regulation and Guidelines development process, and attendees at the webinars, our observation is that these webinars did not represent an impartial presentation of all relevant facts associated at issue in this consultation about public policy.

Phased implementation

As we observed in previous comments, the application of the new additional factors is duplicative and redundant. The basket of comparator countries was amended to include countries that “constrain free market pricing for medicines” through policy measures, i.e. price controls. Therefore, application of the new basket of comparators AND additional factors applies price constraints at least twice. Neither Health Canada nor PMPRB have addressed this issue, or provided a rationale for doubling down on price constraints.

If the goal is to reduce drug prices to below the OECD median, then how far below that benchmark? The comments of PMPRB and the Government of Canada so far have been unclear in this regard.

BMC has taken the position that these regulations and guidelines introduce too many measures at the same time, and some of these measures can be considered experimental having never been tried elsewhere or within the unique Canadian system. Consequently, BMC proposed phasing in aspects of the regulations and guidelines to meet the goal of increasing affordability without additional measures which may have unintended negative impact on patient access to new medications. Specifically, BMC has suggested the application only of the revised basket of comparator companies, and moving the economic factors to a second phase, if necessary.

Modifications to Proposed Guidelines

Improvements

A few of the modifications made to the Guidelines in the latest draft do present some improvements that seem designed to minimize the impact of price reductions on existing medicines, including the use of Highest International Price based on the PMPRB11 basket of comparator countries, as well as the application of the new economic factors only to new medicines. These existing products were brought to Canada under the previous regime, and for public access, faced HTA reviews by CADTH and/or INESSS, and negotiated listing agreements. It should be fair to say that those product agreements reflect the willingness and ability to pay of the public drug plans. Further, the changes to the criteria for “Category 1” drugs have been revised to reduce the number of drug subject to the additional tests. We objected to the absence of meaningful risk assessment. We commented that the criteria for “Category 1” drugs didn’t “seem to do anything other than capture drugs that cost public drug plans a lot of money”.
These changes are improvements because they reduce the number of products subject to the test, but the changes do not address the underlying problems with the approach. Drugs for rare diseases (like PKU) remain as principal targets for price constraints. The greatest risk from our perspective remains that overly restrictive price constraints will delay or prevent new medicines from being launched in Canada and thus harm the health of patients going without these therapies.

**Concerns**

We do have concerns with specific elements of the revisions to the proposed Guidelines. These concerns are generally in areas where we believe the changes have increased the level of arbitrariness, complexity, or uncertainty.

We understand that Guidelines cannot be binding on staff or the Board, but the language that permits staff to “utilize any of the tests described in the Guidelines and modifications or variations of those tests” during an investigation seems to be arbitrary. It would seem reasonable that – in the absence of a clear and compelling rationale, the same standards would apply at introduction and during an investigation. Anything less can be seen as a deviation from the fundamental principles of natural justice.

The application of elements of the Therapeutic Criteria Level (TCL) are particularly confusing. Patient groups made it clear that the therapeutic benefit of a new medicine should be taken into account in value deliberations. In our view, this is one process that should certainly include input from Canadians, something the PMPRB has not done well in the past. The proposal to allow staff to determine the relevant indication should be revisited. A cynic might guess that the Guidelines proposal in paragraph 69 for selecting the relevant indication was selected simply to maximize price constraints. From our perspective, the indication with the greatest therapeutic benefit should be considered relevant. In the absence of useful examples or case studies, it is difficult to determine the utility of this process for patients.

**Conclusions**

Despite the changes to the draft Guidelines, none of our key issues have been acknowledged or addressed. Nothing in the revised Guidelines changes the underlying problems with the Regulations and resulting Guidelines, or the problems with the interpretation and application of the Regulations and Guidelines by PMPRB staff.

These are generational changes that are being applied in a cavalier fashion. The patient communities, including CanPKU, have identified the risks we perceive from the changes. Even with the recent “research” webinars, we do not believe PMPRB or Health Canada have paid sufficient attention to those risks. The new Regulations and Guidelines together mean:

1. Increased uncertainties and threat of low prices will continue to lead to delays in launching in Canada new drugs or decisions not to launch
   This is already already happening in PKU. There has been a failure of the public health system to adopt and fund the initial approved therapy without taking more than ten years. The second and third generations of treatment may avoid Canada altogether.
2. Reduction in clinical trials sponsored by drug developers, and consequently fewer patients gaining early access to promising therapies through clinical trials

3. Combined with provincial coverage policies, patient support programs are jeopardized for specialty and rare disease drugs including for PKU patients, such as:
   - Specialty pharmacy services
   - Infusion services (when drug is infused)
   - Nurse and/or clinical dietician support and patient self-care training
   - Reimbursement navigation including for private payors
   - Co-pay assistance for drug and / or delivery devices (i.e. needles), and
   - Compassionate access for patients with little or no drug insurance (public or private)

These effects will be felt by patients and the health system over the course of years, not months or quarters. Recent evidence, and surveys of business sentiment provide signals of intent, but the real effect will happen over time. If the risks associated with the Regulations and Guidelines do manifest themselves, they will be profound, and will take a concerted effort to change and an equal time to correct. Much like the proverbial frog in a pot, Canadian grassroots patients and policy makers will not perceive the changes happening around them until it is too late.

CanPKU reiterates our comments and recommendations from February 2020 and support the conclusions of BMC. We will offer a few last recommendations for how the Regulatory and Guideline changes can best benefit Canadians and Canadian patients, as follows:

**Recommendations:**

1. The Government of Canada should make a public and explicit statement of its goal(s) for drug price reductions,

2. The Government and PMPRB should phased in the application of the regulations and guidelines to better manage risks of negative impact of these generational changes, starting with only the change in comparison countries.

3. The Government and PMPRB should commit and undertake comprehensive monitoring and evaluation of core metrics, specifically
   - drug launches (number and timing);
   - clinical trials (drugs and patient enrollment); and
   - patient access (time to listing, and provincial variances).
4. The Government and PMPRB should commit to and undertake meaningful processes for patient engagement and participation in decisions.

CanPKU believes it is important that Canada be a market where new treatments for unmet or poorly met needs are brought to market rapidly. We vigorously call for a Canadian drug pricing regime that facilitates the rapid introduction and availability of a comprehensive range of medicines and that provides Canadians the ability to access necessary medicines in a timely manner. Unfortunately, it appears policy makers believe a “last is best, and least risk” is most appropriate for Canada.

PMPRB and Health Canada have repeatedly said that once applied, the Guidelines will result in prices that represent what the public health system is will to pay, is able to pay, and that reflect value for the Canadian Health system. Canadian patient groups like CanPKU don’t believe it, and this is a terrible game of chicken.

We will conclude these comments with the same (unanswered) question we posed in February, 2020. Can PMPRB assure Canadians that any drug sold at the regulated price under these new Regulations and Guidelines will be covered by all public drug plans? Or will Canadians still have to wait, as Canadians with PKU have with Kuvan, for more than ten years and counting?

Thank you for your consideration of this submission.

John Adams
Co-Founder, President & CEO
Canadian PKU and Allied Disorders