Patented Medicine Prices Review Board

Conseil d'examen du prix des médicaments brevetés

Canada

BACKGROUNDER

PMPRB *DRAFT GUIDELINES* CONSULTATION

2019

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The PMPRB *Draft Guidelines* Consultation

The Patented Medicine Prices Review Board (PMPRB) is a quasi-judicial body with a regulatory mandate to prevent pharmaceutical patentees from charging consumers excessive prices during the statutory monopoly period. Its creation arose out of concern that stronger patent protection for medicines might cause their prices to rise unacceptably and become unaffordable to consumers.

Pursuant to subsection 96(4) of the *Patent Act*, the PMPRB issues guidelines ("Guidelines") which are intended to provide transparency and predictability to patentees regarding the process typically engaged in by public servant employees of the PMPRB ("Staff") in seeking to determine whether a patented medicine appears to be priced excessively in any market in Canada. The Guidelines also provide an overview of the processes patentees should be aware of regarding their filing obligations under the *Patented Medicines Regulations* ("Regulations").

On November 21, 2019, the PMPRB announced a 60 day consultation on proposed changes to its Guidelines (the "*Draft Guidelines*"). The purpose of the consultation is to obtain input on the *Draft Guidelines* from patentees, stakeholders and interested members of the public.

Participation in the Consultation

The PMPRB will be consulting with Canadians in the following ways:

- An initial 60 day consultation period during which stakeholders and interested members of the public can provide written submissions on the *Draft Guidelines*
- A policy forum where stakeholders will be invited to appear in person to make their views known to the Board
- The striking of working groups to discuss specific questions and issues arising from the *Draft Guidelines*

The deadline for providing written submissions is January 20, 2020. The timing for the policy forum and technical working groups will be communicated at a later date. The PMPRB is committed to listening to the voices and views of Canadians and looks forward to receiving constructive and meaningful feedback on the new *Draft Guidelines* from this consultation process.

The present Consultation Backgrounder document is intended to be read as a companion piece to the *Draft Guidelines* and provide contextual information on the key changes proposed in that document. Its purpose is to support the reader's understanding of the *Draft Guidelines* and make for a more informed and productive consultation process[1] by providing answers to the anticipated most frequently asked questions hereafter.

1 Only the *Draft Guidelines* are being consulted on. This document is an aid to the consultation process, is not part of the *Draft Guidelines*, and is not being consulted on.

Questions and Answers

1) Why is the PMPRB consulting on new *Draft Guidelines*

Since the establishment of the PMPRB three decades ago, its regulatory environment has changed significantly. In particular, the pharmaceutical industry's research and development focus has shifted from relatively low cost conventional, small molecule drugs for common diseases to high cost biologics and genetic therapies targeted to smaller patient populations. The risk of excessive pricing is often greater for these new products since they have few, if any, competitive substitutes and demand for new and better treatments among the more severely afflicted patient populations is very high. This is especially true for medicines that are first of their kind, or for which alternatives are less effective or have less tolerable side effects.

In addition, it has become common practice for the industry to negotiate confidential rebates and discounts off public list prices in exchange for having their products reimbursed by public and private insurers. This empowers manufacturers to price-discriminate between buyers based on their perceived countervailing power and ability to pay. It also results in a growing discrepancy between public list prices (i.e., gross or "ex factory" prices) and actual prices paid in the market (i.e., net or "average transaction" prices).

Recent amendments to the *Patented Medicines Regulations* (the "Amended Regulations") are intended to provide the PMPRB with the tools and information it requires to carry out its consumer protection mandate effectively in light of these changes. Under the Amended Regulations, the PMPRB can identify gross and net non-excessive ceiling prices of patented medicines in Canada that are more closely aligned with prices in like-minded countries, more reflective of their value to Canadian consumers and more informed by the affordability constraints of the Canadian economy. Changes in the *Draft Guidelines* are necessary to operationalize the regulatory amendments.

2) Changes in the *Draft Guidelines* are necessary to implement amendments to the *Patented Medicines Regulations*. What are these amendments?

The PMPRB determines whether the price of a patented medicine is excessive based on the factors in section 85 of the *Patent Act* (the "Act") using information required of patentees under the Regulations and related data. On August 7, 2019, the Regulations were substantially amended in three main ways:

1. Additional section 85 price regulatory factors

- The amendments supplement the factors the PMPRB is to consider when determining whether the price of a patented medicine is excessive under section 85 of the *Patent Act* to include its value to, and financial impact on, consumers and the health system. The new factors include phamacoeconomic value; market size; and gross domestic product (GDP) and GDP per capita in Canada.

2. An updated schedule of comparator countries (the new "PMPRB11")

- The amendments revise the schedule of comparator countries to be more consistent with the PMPRB's mandate to protect consumers from excessive prices for patented medicines and better reflect current Government of Canada policy priorities.

3. Changes in reporting requirements

- The amendments reduce patentee reporting obligations for patented veterinary, over-the-counter and "generic" medicines so that the PMPRB can focus its attention and resources on patented medicines at greater risk of excessive pricing.
- The amendments require certain patentees to report information relating to the new section 85 factors so that the PMPRB can administer them effectively.

- The amendments require patentees to report price and revenue information net of all price adjustments, including direct and indirect discounts, so the PMPRB's regulatory activities are based on a true and complete picture of patented medicine prices in Canada.

Consequential changes to the Guidelines are necessary to give effect to the Amended Regulations and to formalize the PMPRB's move to a more efficient, risk-based regulatory approach that simplifies and streamlines compliance for patentees.

The publication of *Draft Guidelines* is the latest and final step in a consultative process that dates back to the release of the PMPRB's Discussion Paper on Guidelines Modernization in June 2016 and is in keeping with the roadmap for reform laid out in its 2015–2018 Strategic Plan. Both the *Draft Guidelines* and the Amended Regulations they seek to operationalize will come into force on July 1, 2020.

3) What is new in the *Draft Guidelines?*

As before, the *Draft Guidelines* provide rules of general application which serve as a mechanism for determining how price ceilings will typically be set by the public service employees of the PMPRB (i.e., "Staff"). However, a new approach for monitoring and reviewing patented medicine prices is proposed which includes: 1) an initial screening system for identifying medicines that are likely to be at highest risk of excessive pricing (i.e., "Category I" medicines); 2) separate ceilings for gross or List Prices (the Maximum List Price or "MLP") and Net Prices (the Maximum Rebated Price or "MRP", which will only apply to Category I medicines); 3) price tests which incorporate both the new and old section 85 factors, and 4) a reassessment procedure for medicines that may warrant a higher or lower ceiling because of changes in market conditions. The *Draft Guidelines* also provide guidance to patentees on how to comply with the new filing requirements in the Amended Regulations.

4) What do you mean when you say the *Draft Guidelines* take a risk-based approach to price regulation?

Since June of 2018, the PMPRB has been seeking high level feedback from stakeholders on a new Guidelines framework that would give effect to the Amended Regulations (as pre-published in Part I of the Canada Gazette on December 2, 2017) and support its move to a risk-based approach to regulating the prices of patented medicines that simplifies and streamlines compliance for patentees.

Adopting a risk-based approach enables the PMPRB to make more efficient use of its resources by focusing its regulatory lens on the minority of patented medicines that are believed to be at greatest risk of excessive pricing. This is consistent with the government's policy intent in adding the new section 85 factors and in limiting the requirements for patentees to file cost utility analyses conducted by the Canadian Agency for Health Technology Assessment (CADTH) and l'Institut national d'excellence en santé et services sociaux (INESSS) to patented medicines with annual treatment costs over 50% of GDP per capita. Under the new approach, only those medicines that are screened into Category I would be subject to a comprehensive review under the new factors to determine whether their price is potentially excessive. Medicines at lower risk of being excessively priced would fall into Category II and face relatively less oversight.

5) How do the *Draft Guidelines* simplify and streamline compliance for patentees?

The key difference between the current Guidelines and the *Draft Guidelines* is that, once set, the PMPRB's ceiling prices do not fluctuate from year to year depending on the net or average transaction price (ATP) of the patented medicine in the previous year. This is an onerous rule that requires patentees to keep careful track of yearly ATPs and adjust their prices accordingly to remain in compliance. Under the *Draft Guidelines*, patentees have complete freedom to vary their pricing from year to year, provided it remains below the Maximum List Price (MLP) and Maximum Rebated Price (MRP), as applicable. In addition, the majority of patented medicines will fall into Category II for which compliance with the MLP will be assessed solely based on list prices. The *Draft Guidelines* also substantially reduce the number of markets and customer classes for which patentees are required to file price information and now focus only on national and provincial or territorial pricing. As a result, patentees are no longer required to file separate information related to sales to hospitals, pharmacies and wholesalers. As compared to the previous *Compendium of Policies, Guidelines and Procedures*, this streamlined approach is reflected in a higher level *Draft Guidelines* document that is more succinct, less technical, and explains the PMPRB's regulatory framework in a manner that is much easier for patentees and stakeholders to understand.

6) What are the new s.85 factors and how has the PMPRB interpreted them in the *Draft Guidelines*?

The Amended Regulations add three new factors to subsection 85(1), namely pharmacoeconomic value, market size, and gross domestic product (GDP)/GDP per capita in Canada. The RIAS indicates that the introduction of these factors arises from the realization that unit price alone does not provide sufficient context by which to evaluate excessiveness in today's regulatory environment. Specifically, price divorced from cost to consumers does not capture key inputs in determining whether a medicine represents good value for money relative to the technologies it will displace or what its broader impact may be on the ability of the health system to absorb additional such technologies. These are critical considerations in an era marked by increasingly constrained health budget envelopes, an aging population and a burgeoning number of drugs with annual average treatment costs in the hundreds of thousands of dollars.

7) What does pharmacoeconomic value mean in the context of the *Draft Guidelines*?

Pharmacoeconomic value is a measure of how much a drug costs for the health benefit it provides, which can be compared to other drugs or health technologies by using a standard measure of benefit. The standard measure preferred by health technology assessment (HTA) agencies worldwide is the Quality Adjusted Life Year (QALY). Evidence of the expected costs and health effects of making a new health technology available to specific populations in a particular setting and health

care system are often summarized as incremental cost-effectiveness ratios (ICERs) and expressed as the cost per QALY gained. ICERs provide a useful metric for quantifying how much additional resources are required to achieve a measured improvement in health (i.e., the additional cost required to gain one QALY).

In a public health care system, a new health technology will only improve health outcomes overall if its additional health benefits exceed the opportunity costs associated with the additional resources required to pay for it. Opportunity cost is measured by reference to the estimated health foregone by other patients within the health care system when fixed and fully allocated resources are used to adopt a new medicine. Such an assessment of health opportunity cost reflects the maximum a health care system can pay for the health benefits that a new health technology offers without reducing total population health. This is referred to as a supply-side threshold and requires knowledge of the marginal cost of a QALY within that health system (i.e., the point at which spending on a new health technology for one set of patients in the public system will result in the loss of one QALY for another set of patients in the system).

It is often noted that Canada is the only country with a publicly funded health care system that does not include universal pharmaceutical coverage. The result is a patchwork of public and private payers who lack the national buying power to counter the statutory monopoly position of patentees. That monopoly position is bolstered when an ever-increasing proportion of public and private spending is taken up by high cost drugs with few or no direct competitors. By requiring that the PMPRB consider the pharmacoeconomic value of these drugs, the Amended Regulations ensure that the concept of opportunity cost is taken into account in determining whether their price is excessive. Given that the private market for pharmaceuticals in Canada is an offshoot of the public system and cannot function without it, the policy intent in the Amended Regulations is for the PMPRB to adopt the perspective of the public health care system and favour a supply-side cost effectiveness threshold in estimating opportunity cost.

8) What does market size mean in the context of the *Draft Guidelines*?

The economic impact of a particular drug on consumers is a function of both price and volume. Where public and private insurers are called on to cover the cost of a medicine for a significant number of patients, its price could render it unaffordable to consumers. This can be true even of medicines with favorable pharmacoeconomic profiles because their large market size can eventually result in the displacement of more cost effective technologies and contribute to unsustainable growth in health care costs. The converse is also true of medicines with a very small market size in that they do not tend to raise affordability constraints on a one-off basis even when they have a very high opportunity cost. By requiring the PMPRB to consider the size of the market for a medicine, the Amended Regulations ensure that the global impact on pharmaceutical expenditures of paying for the medicine for everyone who needs it is taken into account in determining if its price is excessive. They also allow the PMPRB to reassess the prices of patented medicines over time, as their market size expands or contracts.

9) What does GDP and GDP per capita mean in the context of the *Draft Guidelines*?

Gross Domestic Product (GDP) is a measure of a country's economic output. GDP growth measures how much the inflation-adjusted market value of goods and services produced by an economy is increasing over time. Per capita GDP measures how much a country is producing relative to its population. The former is looked at as an indicator of societal wealth while the latter is looked at as indicator of level of economic development within that society.

While it is recognized that the financial circumstances of different institutional consumers in Canada will vary, year over year growth in GDP serves as an indicator of what the entirety of the Canadian population can afford to pay for the new patented

medicines that come to market on an annual basis. Per capita GDP can serve a similar purpose as a proxy for what would be considered excessive for individual consumers. The addition of these factors enables the PMPRB to assess the economic impact of a patented medicine's price on both insurers and individual consumers and enable it to develop screening criteria and market size tests for medicines that are likely to pose affordability challenges for the Canadian health care system.

10) How did the PMPRB arrive at a $60,000/QALY pharmacoeconomic value threshold in the *Draft Guidelines*?

The $60,000/QALY threshold is based on preliminary academic work commissioned by the PMPRB to calculate the marginal cost of a QALY in the Canadian health system, empirical estimates of supply-side thresholds from other relevant jurisdictions, and evidence of historical trends in cost effectiveness assessments by Canadian HTA agencies.

In the Canadian health system, the federal government administers national standards and provides financial support to the provinces and territories who are responsible for managing and delivering health care services. The supply-side threshold for any specific province or territory is a function of its individual health care system budget, among other factors. An assessment of health opportunity costs for the Canadian health system commissioned by the PMPRB (Ochalek, Lomas, and Claxton, 2018) concluded that a $30,000 cost per QALY would be reasonable for Canada as a whole and is likely to be similar across most provinces. This is broadly in line with empirical estimates of supply-side thresholds in other jurisdictions with similar wealth and pharmaceutical market characteristics as Canada.[2]

In the final report of the *Working Group to Inform the Patented Medicine Prices Review Board Steering Committee on Modernization of Price Review Process Guidelines* (the "Working Group"), it was observed that no Canadian HTA agencies currently specify an explicit cost per QALY policy threshold. However, anecdotal evidence exists of informal policy thresholds of between $50,000 and $100,000, with oncology medicines typically at the higher end of the range.[3]

In giving effect to this new factor in the *Draft Guidelines*, the PMPRB must contend with the relatively embryonic state of empirical work on a supply-side cost effectiveness threshold in Canada. It must also account for the inherent uncertainty in ICER values and the cost utility analyses upon which they are based. The PMPRB's role as a ceiling price regulator, not a price setter, and the continuing imperative of providing Canadian patients with access to effective new heath technologies has informed its approach to addressing these contextual considerations. Accordingly, the *Draft Guidelines* propose an initial cost effectiveness threshold of $60,000 per QALY, which is two times the current best estimate of the marginal cost of a QALY in the Canadian health system. The threshold will be recalculated periodically to reflect changes in GDP, the size of health care budgets, advancements in empirical research, and the marginal productivity of health care services that face displacement from the adoption of new medicines.

2 This includes: work by Claxton et al. (2015), which estimated a supply-side threshold of £12,936 per QALY for the public health care system in the UK; work by Vallejo-Torres et al. (2017), which estimated a supply-side threshold of between €21,000 and €25,000 per QALY for the public health care system in Spain; and work by Edney et al. (2017), which estimated a supply-side threshold of AU$28,033 per QALY for the public health care system in Australia.

3 A 2016 article in the Hamilton Spectator reported that "the pan-Canadian Oncology Drug Review has set an unofficial threshold of $100,000 per quality-adjusted life year for new cancer medications". A 2009 letter by the Deputy Minister of Health and Long-Term Care for Ontario noted that the Committee to Evaluate Drugs "typically considers a range of $40–60,000 [per] QALY as an acceptable range".

11) How did the PMPRB arrive at the $25 million market size threshold in the *Draft Guidelines*?

In addition to applying a $60,000 cost per QALY pharmacoeconomic value threshold, the *Draft Guidelines* provide that the MRP for Category I medicines may be further adjusted if the product's annual revenues exceed a threshold of $25 million. As mentioned, the amendments adding the new market size and GDP factors to the Regulations recognize the tension that can arise between a medicine's long-term value for money (i.e., its cost effectiveness) and its short-term affordability within the health system. The following assumptions were used to guide the calculation of the $25 million affordability threshold, which is based on the combined application of these two factors:

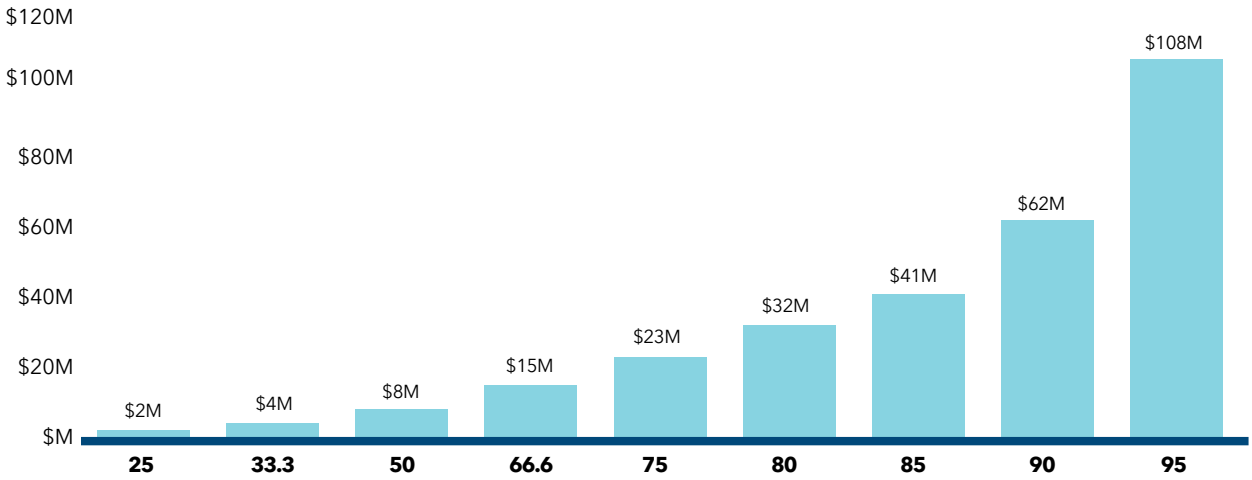
1. To be sustainable, new spending on patented medicines should be commensurate with anticipated annual growth in GDP in Canada;
2. As expenditures for health care increase in proportion to GDP growth, the amount of health care spending attributable to patented medicines should remain the same;
3. The number of new patented medicines anticipated yearly is an average of the preceding 5 years;
4. An average national net budget impact can be calculated for each new patented medicine that would absorb the budget envelope available for all new spending on patented medicines within the GDP growth estimate;
5. The average national net budget impact is not an appropriate market size threshold because too many drugs would have a potential budget impact greater than the average;
6. A threshold set at double the average national net budget impact is more in keeping with a risk based approach as it focuses regulatory attention on those patented medicines that are most likely to have an excessive opportunity cost because of their market size.

A growth in the sales for the patented medicine in Canada that would be in line with the above assumptions would result in an annual increase in patented medicine sales of $432M. Considering that there are on average 36 new patented medicines introduced every year to absorb this growth, it would mean that each medicine would generate on average $12M in patented drug sales, as shown below. The $25 million threshold is approximately two times that amount.

Average annual patented medicines sales, 2014 to 2018 ($B)	**$14.9B**
Average annual growth in patented medicine sales, in line with the growth rate in GDP	**$432MB**
Average number of new patented medicines introduced per year, 2014 to 2018	**36**
Average annual affordability threshold per medicine	**$12M**

PMPRB analysis indicates that over 75% of the patented medicines introduced during the last 20 years realized revenues below this threshold in any of their first three years of sale.

Percentiles analysis of patented medicines by maximum revenues by 3rd year (639 medicines introduced since 1998)



Source: PMPRB

This number will be updated periodically to account for changes in the CPI and in GDP.

12) Why do the *Draft Guidelines* allow Category I medicines that treat conditions with very low prevalence in Canada a 50% percent increase in the MRP?

Increasing awareness and technological advances means that more Canadians are being diagnosed with and seeking treatment for rare diseases and disorders. Consistent with the Government of Canada's commitment in Budget 2019 to support Canadians with rare diseases, the *Draft Guidelines* include a MRP adjustment for patented medicines for rare diseases and disorders with small patient population (i.e., small market size due to low prevalence). The adjustment effectively means that ceiling prices for the Net Price of patented medicines for rare diseases and disorders realizing small volumes of sales will be higher than those for more common conditions. This also allows for the even application of the pharmacoeconomic value factor across all Category I patented medicines (i.e., using the same $60,000 per QALY threshold) while at the same time resulting in substantially higher MRP ceilings for medicines for rare diseases and disorders.

The PMPRB has set the threshold for a rare disease or disorder at any disease that is found in less than 1 in 2,000 Canadians. This measure is based on the definition used by the Canadian Institutes of Health Research, Health Canada, the European Union, The February 2019 Report of the Standing Committee on Health on Canadians Affected by Rare Diseases and Disorders, and Canadian patient organizations.

Under the *Draft Guidelines*, patented medicines for rare diseases and disorders will see their MRP increased by 50%, as long as their annual revenues fall within the $12.5M average annual affordability threshold defined above. This 50% premium is intended to reflect the prevailing revenue premium these medicines currently realize over non-rare disease medicines. The MRP will then be applied incrementally to any sales in excess of this threshold.

The MRP is set at a premium of 50% over the pharmacoeconomic price (PEP) for the revenues up to $12.5M. For revenues ranging from $12.5M to $25M, the MRP is set to the PEP,

while for revenues in excess of $25M the MRP is set in accordance to the market size adjustments for other Category I medicines. This premium is supported by the data analysis of medicines introduced between 2010 and 2015, which have a patient population that is less than 1 in 2000 Canadians, and for which a PEP could be derived. The results suggest that, on average, the price required for these medicines to realize $12.5 million would need to be 49% over the PEP.

13) Why do the *Draft Guidelines* not use the same cost per QALY thresholds mentioned in the cost benefit analysis (CBA) section of the RIAS?

The RIAS includes a CBA section which describes the calculation of the expected economic impact of the newly prescribed section 85 factors. The CBA is based on a model that made a number of assumptions, including that the PMPRB would apply a $50,000 cost per QALY threshold for medicines that treat standard diseases, a $150,000 cost per QALY threshold for medicines for rare diseases and a $35,000 cost per QALY threshold for medicines that treat indications with a high-prevalence in the Canadian population. These assumptions are offered up as one possible implementation scenario of the new section 85 factors for modeling purposes and are not intended to serve as mandatory thresholds that the PMPRB must abide by in its Guidelines. The PMPRB's use of a $60,000 cost per QALY threshold for all medicines and its allowance of a 50% increase in the MRP for medicines for rare diseases and disorders is consistent with the overarching policy intent of the Amended Regulations.

14) What are the new reporting requirements in the Amended Regulations?

The Regulations specify what information patentees must provide to the PMPRB about the prices of patented medicines sold in Canada and other countries, patentees' revenues and R&D expenditures. The Amended Regulations require patentees to report additional information that is relevant to the new section 85 factors, with the exception of information related to GDP and GDP per capita, as this would be obtained from Statistics Canada.

As regards pharmacoeconomic value, the Amended Regulations require that patentees of medicine's with an annual cost above 50% of GDP per capita to provide the PMPRB with all published cost-utility analyses for medicine as reported by a publicly funded Canadian HTA agency, namely CADTH or INESSS. The PMPRB will not duplicate the pharmacoeconomic analysis undertaken by CADTH and INESSS in producing them.

The requirement to provide the most recently published cost-utility analysis received by the patentee applies only to Category I medicines with an annual cost above 50% of GDP per capita. Cost-utility analyses are typically only prepared at certain key points in a medicine's life cycle (e.g. prior to initial market launch or following regulatory approval for a new indication). There is no obligation on patentees to prepare a cost-utility analysis if a publicly reported one does not exist. Where a cost-utility analysis is not available, the MRP will be calculated using alternative information, such as the prices of medicines in the same therapeutic class, international price information, and market size information.

As regards market size, the Amended Regulations require patentees to provide the PMPRB with information on the estimated maximum use of the patented medicine in Canada by quantity of the medicine sold; and with information about the expected timing of the maximum use. Patentees already compile this type of information in the development of business plans, sales forecasts and for CADTH processes. Patentees would also be required to provide the PMPRB with any updated estimates that may result from the approval of a new indication or some other significant change in the market for the medicine.

15) Why does the MRP only apply to Category I patented medicines?

Under the *Draft Guidelines,* Category I medicines are subject to both a List Price ceiling (MLP) and a Net Price ceiling (MRP). Category II patented medicines are only subject to a List Price ceiling (MLP).

The application of an additional ceiling (MRP) to Category I patented medicines is based on (a) the greater likelihood that these patented medicines will have few or no therapeutic comparators and would put consumers (i.e., public and private insurers) at a significant disadvantage in seeking to negotiate a socially acceptable price with the patentee; and (b) the greater likelihood that the market size for these patented medicines could raise affordability concerns.

16) Why is the MRP assessed against the Net Price as opposed to the List Price?

The assessment of the MRP against the Net Price is intended to reflect the reality of confidential pricing in the context of Category I patented medicines and to facilitate the patentees' ability to meet the ceilings in the *Draft Guidelines* without affecting the public List Price.

17) Why is the MLP assessed against the List Price as opposed to the Net Price?

The assessment of the MLP against the List Price as opposed to the Net Price allows for an "apples to apples" comparison where the tests being used to set the MLP are all based on domestic and/or international list prices, which do not include rebates.

18) What confidentiality provisions apply to patentee information under the Patent Act?

The Act provides for the confidentiality of information supplied to the PMPRB in certain circumstances. Specifically, information or documents provided to the PMPRB in accordance with the provisions dealing with pricing information in sections 80, 81 and 82 of the Act (e.g. revenues, average price or net revenues and associated discounts and rebates) or in any proceeding relating to excessive prices under section 83, is privileged and cannot be disclosed to the public without authorization of the disclosing party, unless such information has been disclosed at a public hearing under section 83 of the Act or is subject to the exceptions outlined in section 87(2) of the Act. Information provided to the PMPRB may also be subject to certain provisions in the *Access to Information Act* and the *Privacy Act*.

19) Why do the *Draft Guidelines* only require patentees to submit a cost utility analysis for medicines with an average annual treatment cost of more than 50% of GDP per capita?

This is stipulated in section 4.1(5) of the Amended Regulations.

20) Why does the PMPRB use the CADTH report as the primary source model?

There are several potential sources for the cost-utility analysis patentees are required to file for Category I medicines. These are the Common Drug Review (CDR) Pharmaceconomic Reports and pan-Canadian Oncology Drug Review (pCODR) Final Economic Guidance Reports of CADTH and the models developed by INESSS in its *Évaluations aux fins d'inscription*.

The PMPRB's default approach will be to rely on CADTH for its primary source models because they are representative of a larger share of Canada's population. Where CADTH reports are unavailable or do not meet the criteria defined in the guidelines (e.g. because they do not feature a cost-utility or cost-minimization modeling approach), INESSS reports will be used.

21) Why do the *Draft Guidelines* provide for a single price ceiling for a patented medicine with multiple indications?

Following consultation with stakeholders and technical experts, the PMPRB is of the view that indication-specific pricing would be extremely difficult to implement in Canada at this time, given current limitations in data capture and reporting on volume per indication. In view of these considerations, the *Draft Guidelines* adopt the simpler approach of a single price ceiling across multiple indications. In cases where a patented medicine has more than one indication, the PMPRB will identify the indication likely to have the greatest impact on consumers (i.e., the "Relevant Indication") and will set its ceilings on that basis. For Category I medicines, this will be the indication triggering the Category I screening criteria; for Category I medicines where more than one, or no, indications meet this threshold, and for Category II medicines, the Relevant Indication will be the indication treating the condition with the highest prevalence (i.e., the largest patient population).

22) Will the PMPRB work with CADTH and INESSS to ensure that the necessary data appears in their reports?

The PMPRB recognizes that CADTH and INESSS have dedicated expertise and a reputation for excellence in the generation of comprehensive HTA reports in Canada. The PMPRB does not intend to duplicate any of the work performed by CADTH and INESSS in this regard.

The PMPRB will work collaboratively with CADTH and INESSS on any modifications that may be required to their processes and reports to satisfy the requirements of the new regulatory framework.

23) Why are grandfathered patented medicines subject to the new Schedule of comparator countries and why does the international median set their ceiling price?

Transitional provisions in the Amended Regulations grandfather patented medicines that received a Drug Identification Number (DIN) from Health Canada prior to the publication of the Amended Regulations in Canada Gazette Part II on August 21, 2019, from the application of the new section 85 factors but not the new Schedule of comparator countries we are now calling the PMPRB11. Patented medicines sold in Canada under the Special Access Program (SAP)

prior to August 21, 2019 will not be grandfathered because SAP medicines are not assigned a DIN. In addition, line extensions of existing products that are assigned a DIN after August 21 2019 are not grandfathered even though the original related products existing prior to August 21, 2019 are.

The MLP for grandfathered medicines is set at the lower of (i) the median price for the PMPRB11 countries for which the patentee has provided information, or (ii) the ceiling price which had been in effect prior to the issuance of these Guidelines. The median ceiling price is considered to be consistent with Canada's responsibility to pay its fair share for global biopharmaceutical innovation. The MLP may also be adjusted by CPI in certain circumstances.

24) How is CPI addressed in the *Draft Guidelines*?

Paragraph 85(1)(d) of the Act requires the PMPRB to consider changes in the consumer price index (CPI) when determining whether the price of a patented medicine is excessive. In the context of the *Draft Guidelines*, this factor is addressed in three ways: (1) the MLP is set within three years of introduction, which mimics the effect of allowing a CPI increase as compared to the average rate of decrease in international prices, if these prices continued to be used to set the MLP annually; (2) where the MLP is set by the median international prices and this median increases over time, the MLP may be adjusted on the basis of CPI; and (3) the affordability threshold will be updated periodically to account for changes in the CPI. This ensures that the *Draft Guidelines* tests and ceilings remain relevant in the light of changes in the Canadian economy while also protecting Canadians from paying higher prices for patented medicines than consumers in the PMPRB11.

25) How and when will the PMPRB monitor and report on the impact of the new Regulations and Guidelines and whether they are working as intended?

The PMPRB has committed to developing and conducting an extensive monitoring strategy, the Guidelines Modernization and Evaluation Process (GMEP), to track the impact of the Guideline changes on patients, health care providers, and other stakeholders.

The GMEP will focus on the following key areas: 1) Impact on Medicine Prices, 2) Impact on Medicine Access, 3) Impact on the Economy; and 4) Impact on PMPRB processes. For all areas, the PMPRB will use administrative, commercial and internal data sources to investigate and document changes after implementation of the Guidelines relative to the before their implementation. Although the majority of the indicators will be quantitative, the GMEP will also employ a number of qualitative indicators.

A detailed GMEP proposal, including the scope of the reporting that will be made publicly available, will be presented to the PMPRB Board for approval in early 2020. The GMEP plan will be made publicly available, and a first GMEP report will be published after at least a full year of post-implementation data are available for analysis. In subsequent years, periodic reports will be published to monitor long-term impacts.

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Medicine Prices
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