

WRITTEN FEEDBACK TO
PATENTED MEDICINES PRICE REVIEW BOARD
DRAFT GUIDELINES CONSULTATIONS
2020

ON BEHALF OF

Auto Sector Retiree Health Care Trust (asrTrust)

Canadian Union of Public Employees
Education Workers' Benefits Trust (CUPE EWBT)

Elementary Teachers' Federation of Ontario
Employee Life and Health Trust (ETFO ELHT)

Ontario English Catholic Teachers Association
Employee Life and Health Trust (OECTA ELHT)

Ontario Non-union Education Trust (ONE-T)/
Fiducie des employées et des employés
non-syndiqués en éducation de l'Ontario (FENSÉO)

Ontario Secondary School Teachers' Federation /
Fédération des enseignantes-enseignants de l'Ontario
Employee Life and Health Trust
(OSSTF/FEESO ELHT)

Retired Teachers Insurance Plan ("RTIP")

Prepared by
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Aug 3, 2020

Doug Clark

Executive Director

PMPRB-CEPMB

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Re: **Comments on revised guidelines** for 30-day consultation period

Mr. Clark,

On behalf of a group of Ontario-based trusts responsible for active teacher and education sector employees, retired auto sector workers, and retired teachers and education workers in the province of Ontario, we are happy to provide these comments in response to your 30-day consultation period on the revised PMPRB-CEPMB ('PMPRB') guidelines.

We have based our new comments on the revised PMPRB guidelines and how our initial comments appear to have been addressed. In short, we agreed with the stated intent of the previous set of guidelines but felt they harbored several key shortcomings including but not limited to:

- *QALYs* - The prescriptive use of a quality-adjusted life-year (QALY) to consider economic value may be too constrained and not reflect broader elements of value to patients
- *Affordability*
 - The price thresholds proposed for Category 1 drugs led to annual drug costs that are still unaffordable to consumers- contrary to the stated intent of the PMPRB.
 - To this end, drugs that are considered “expensive” by private drug plans are typically \$10K--- much less than GDP-based thresholds proposed by the PMPRB.
- *Two-tiered pricing* - The regulation of MLP/MRP leads to two-tier pricing that is unfair to those with private drug plans or paying out-of-pocket.
- *Value – based pricing*
 - *Price referencing* - Continued use of international price referencing does not lead to prices that reflect the underlying value of commercialized products.
 - Floor prices do not reflect the costs of production by manufacturers.

- The incremental cost per QALY threshold proposed did not reflect available empirical evidence and seems to reflect PMPRB capacity and potential underfunding towards adequate price regulation rather than curbing excessive pricing.
- *Price based on prevalence of illness*
 - There is a premium based on drugs of very low prevalence that does not reflect an abundance of evidence that demonstrates Canadians consider age and suffering of patients as relevant measures for priority setting, not disease prevalence.
 - The threshold chosen for very low prevalence was out of line with international jurisdictions, with a global average of 1/2500.

With the recent revisions to the Guidelines we have had the opportunity to explore how the PMPRB has addressed the concerns of our group, which is responsible for covering over 740,000 individuals in the province of Ontario, and, that we suspect reflects similar concerns from plan sponsors in other parts of the country (although we don't represent them). While some changes have been implemented that help address some of our initial concerns, we would note the following:

- *QALYs – no change and further concerns*
 - It seems the QALY is still being promoted as an outcome measure with no regard for contextual circumstances that may include measures of benefit beyond length and health-related quality of life. This could include benefits to caregivers, benefits to the health system in other ways, or benefits beyond the health system.
 - With the introduction of the National Advisory Committee on Drug Immunization (NACI) as a source of economic evaluation, the guidelines fail to recognize that economic evaluations in vaccination typically adopt a societal approach¹ and may use outcomes other than QALYs--- typically disability adjusted life-years (DALYS – recommended by the World Health Organization²) but also benefit cost ratios– consistent with World Health Organization guidance and the Erickson De Wals framework adopted by the NACI.

¹ L. J. Erickson, P. De Wals, and L. Farand, “An Analytical Framework for Immunization Programs in Canada,” *Vaccine* 23, no. 19 (March 31, 2005): 2470–76, <https://doi.org/10.1016/j.vaccine.2004.10.029>.

² “WHO-IVB-19.10-Eng.Pdf,” accessed August 3, 2020, <https://apps-who-int.proxy.bib.uottawa.ca/iris/bitstream/handle/10665/329389/WHO-IVB-19.10-eng.pdf>.

- *Affordability – no meaningful change*
 - The annual treatment cost threshold for high priced drugs still ignores what is actually affordable to the Canadian consumer. A 150% GDP/per capita annual price still amounts to a roughly ~\$90,000 annual cost. This is likely unaffordable to an out-of-pocket consumer or even a consumer with insurance and a 20% co-payment.
- *Two-tiered pricing – no change and concerns*
 - The new version of the guidelines still creates two-tiered pricing for those with and without public insurance. Our plan members will still be subject to maximum list prices for which insurers may or may not receive rebates and who have no obligation to pass on the savings.
 - We are also curious how the new MRP guidance might be enforced, given the recent ruling that held that the New Price Calculation may be unlawful, void and of no force and effect because it extends beyond sales made by the patentee at the factory-gate.³
- *Value – based pricing*
 - There have been no changes to the International Price Referencing provisions. Price referencing in this way ignores the value of products to Canadians, and is subject to gaming and market failure. In particular, price referencing inherently contributes to an international price configuration that may incent private sector producers of new drugs to delay or accelerate introductions to Canada in order to optimize profits in ways that are difficult to predict.
 - Similarly, there has been no added provision for addressing manufacturer floor prices. Like price referencing, this could create conditions for market failure and make access to new drugs unpredictable.
 - Rather than reduce the ICER threshold to a value that reflects supply-side constraints empirically shown in Canada, the ICER has been raised. This could further contribute to lost opportunities for health by Canadian patients, as resources are unfairly committed to new drugs for less benefit than would have otherwise been realized by investing them in other parts of the health system.

³ Smart & Biggar, “Federal Court Strikes Price Calculation Provision of Amendments to Patented Medicines Regulations,” Smart & Biggar, accessed August 3, 2020, <https://www.smartbiggar.ca/insights/publication/federal-court-strikes-price-calculation-provision-amendments-patented-medicines-regulations>.

- The new exemption for specialty drugs with annual costs < \$12M also unfairly allows for price premiums for drugs with low prevalence (regardless of value) although we are happy to see that a special emphasis on disease prevalence *per se* has been omitted.

In short, while we appreciate the opportunity to respond to the newly revised guidelines, we are disappointed to see that the changes made do not reflect our concerns that they did not go far enough to regulate prices *nor* likely the concerns of the many Canadians who rely on sponsored plans with constrained (or even, fixed) budgets that cannot be easily or quickly increased. This is even of significance to Canadians without plans, for which annual out-of-pocket expenditures would fall far short of *affordable drugs for Canadians*, a stated objective of the PMRPB.

Instead, the new revisions seem to relax previous proposals to constrain excessive prices, making drugs even *less affordable* than in the previous version of proposed Guidelines, and introduce new rules such as the use of QALYs based on the economic evaluations provided by the NACI, or the use of confidential price rebate information, which are likely not tenable.

In total, this gives the impression that rather than consider the impact to *actual* Canadians, and introducing constraints that will lead to *affordable medication and sustainable expenditure increases*, the PMPRB has chosen to listen to the many private companies which the PMPRB was originally intended to govern. Namely those with special patent protections, who still enjoy supernormal profits and whose sustainability, unlike the Canadian health care system, is not threatened by these measures.

Respectfully,

Gordon Graham, asrTrust

Corina Feiger, ONE-T/ FENSÉO

Salama Rajab, CUPE EWBT

Donna Morrison, OSSTF/FEESO
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Suzanne Gill, ETFO ELHT

Darryl Ingham, RTIP

Dan Boucher, OECTA ELHT

APPENDIX 'A'

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Ontario Teachers Insurance Plan (OTIP)

Retired Teachers Insurance Plan ("RTIP")

Prepared by
Don Husereau, BScPharm, MSc

CONTEXT - ABOUT US

We represent trusts responsible for active teacher and education sector employees, retired auto sector workers, and retired teachers and education workers in the province of Ontario. Our organizations sponsor private insurance plans that provide supplementary health benefits coverage, including for patented and generic medicines, to individual plan members and their dependents.

- The asrTrust provides benefits to over 70,000 Ontario auto sector retirees based on a one-time fixed contribution. There will be no new contributions to this fund as it was established by former employers. Funding was one-time only and must last for the life of the Trust...over 60 years.
- The CUPE EWBT provides health benefits to about 46,800 CUPE members in the education sector in Ontario. Inclusive of members, spouses and dependents, approximately 114,000 are enrolled in the CUPE EWBT benefits plan.
- The ETFO ELHT has two divisions (teacher and education worker) and provides health benefits to approximately 65,000 members of the Elementary Teachers' Federation of Ontario (ETFO). Inclusive of members, spouses and dependents, the ETFO ELHT has approximately 185,000 eligible claimants enrolled in the teacher and education worker plans.
- The OECTA ELHT has four separate divisions and provides health benefits to approximately 42,000 members of the Ontario English Catholic Teachers' Association (OECTA); 6,400 members of the Ontario Council of Educational Workers (OCEW); 3,950 members of the Education Workers' Alliance of Ontario-Alliance des travailleuses et des travailleurs en education de l'Ontario (EWAO-ATEO) and 650 members of Unifor all of whom are employed in the education sector in Ontario. Inclusive of members, spouses and dependents, the OECTA ELHT has approximately 136,746 eligible claimants enrolled in the various divisions of the plan.
- The Ontario Non-Union Education Trust (ONE-T) provides benefits to non-union employees in the Ontario education sector. The trust provides benefits to approximately 7,500 management and non-union employees, and 7,500 Principals/Vice-Principals (P/VPs). ONE-T's total membership including dependents is about 50,000 members, creating a solid framework for a strong and sustainable benefits trust.

- The OSSTF ELHT has two divisions providing health benefits to approximately 30,000 teachers and 16,000 education workers of Ontario School Teachers Federation of Ontario (OSSTF). Inclusive of members, spouses and dependents, OSSTF Benefits has approximately 123,000 eligible claimants enrolled in the teacher and education worker healthcare plans
- The RTIP serves 36,000 primary members with 28,200 dependents – for a total of 64,800 individuals. As a retiree population, its plan members have specific drug therapy issues related to aging including new limited additional benefit medications that are significantly more expensive in treating high prevalence conditions such as diabetes.

Taken together, we are responsible for covering over 740,000 individuals in the province of Ontario. As plan sponsors for retirees, teachers, and other education workers in Ontario, we are deeply concerned about increasing prices and overall expenditures for new medicines. Our plans have constrained (or even, fixed) budgets that cannot be easily or quickly increased:-

In some cases, as with retiree plans, costs cannot be passed along to members who have constrained incomes. While 6.4% year-to-year increases⁴ (and > 10% in some of our plans) may be affordable to some, they are unsustainable to our plan members in an environment where higher-priced drugs are a more important cost driver than an aging demographic. We have seen expensive drug claims for between 1 - 3% of our plan members account for 30 - 65% of annual spend consistent with the public sector.⁵ Therefore, we appreciate the PMPRB's renewed focus on expensive medications.

We also rely on the existing market of private insurance carriers for reimbursement of patented medicines and other healthcare goods and services. While we are aware that the focus on rebated prices for expensive drugs may benefit the private insurance industry, and indirectly benefit our plan members (through regulating rebated prices, reducing payers potential losses and allowing them to charge affordable premiums), we need the PMPRB to understand that expensive and unaffordable drugs are still a direct threat to individual plan members who are subject to plan maximums or co-payments. They are also a threat to our plans as expensive claims make it harder to maintain other existing benefits. We will elaborate on these points below as we feel they will be useful to the PMPRB.

⁴ "Poster 7 - 2018 Cost Drivers of Private Drug Plans in Canada, 2017," accessed January 15, 2020, <http://www.pmprb-cepmb.gc.ca/view.asp?ccid=1372&lang=en>.

⁵ "CIHI : Prescribed Drug Spending in Canada, 2019: A Focus on Public Drug Programs," n.d., <https://www.cihi.ca/sites/default/files/document/pdex-report-2019-en-web.pdf>.

GENERAL COMMENTS

In formulating our response to the Draft Guidelines, we will address the questions asked at the Dec 10, “Civil Society Forum” presentation.⁶ We will then provide some specific commentary and examples based on our collective experience.

1. What aspect(s) of the proposed Guidelines do you agree with and why?

We agree with the stated intent of the guideline, which is to provide a consistent and transparent process for the PMPRB to carry out its consumer protection mandate and ensure prices of patented medicines are not excessive. We recognize the high costs of medicines can have a direct impact on patients by leading to cost-related non-adherence and poorer outcomes for patients.⁷

We also agree that prices are intended to reflect additional benefits to consumers (and additional costs to suppliers) and recognize the imperfect market conditions under which patented medicines are sold, including the lack of competition, which makes some form of pricing framework necessary. As the economist Robert G. Evans once stated, “Rising health costs are not a law of nature, like the tides. They are responsive to well-crafted policy”.⁸

Cost-effectiveness measures

The use of quality-adjusted life-years to establish a measure of benefit is a conventional, long-standing, and well-accepted starting point.⁹ We concede that important domains of health-related quality of life (e.g., pain, function) coupled with longevity are important goals for those taking patented medicines. However, we hope the PMPRB recognizes that other measures of mortality adjusted for measures of preferences for health, such as the disability adjusted life-years, are currently promoted in International Guidance, such as for vaccines.¹⁰

We also hope the PMPRB recognizes the Canadian Agency for Drugs and Technologies in Health (CADTH) and Institut national d’excellence en santé et en services sociaux (INESSS) Guidelines are not strict about methods to estimate

⁶ “Presentation-Dec10-En.Pdf,” accessed January 17, 2020, <https://www.canada.ca/content/dam/pmprb-cepmb/documents/consultations/draft-guidelines/presentation-dec10-en.pdf>.

⁷ Michael R. Law et al., “The Effect of Cost on Adherence to Prescription Medications in Canada,” *CMAJ*: *Canadian Medical Association Journal* 184, no. 3 (February 21, 2012): 297–302, <https://doi.org/10.1503/cmaj.111270>; Michael R. Law et al., “The Consequences of Patient Charges for Prescription Drugs in Canada: A Cross-Sectional Survey,” *CMAJ Open* 6, no. 1 (February 5, 2018): E63–70, <https://doi.org/10.9778/cmajo.20180008>.

⁸ Robert G. Evans, “The TSX Gives a Short Course in Health Economics: It’s the Prices, Stupid!,” *Healthcare Policy* 6, no. 2 (November 2010): 13–23.

⁹ “Critical Ratios and Efficient Allocation,” *Journal of Public Economics* 2, no. 2 (April 1, 1973): 147–57, [https://doi.org/10.1016/0047-2727\(73\)90002-9](https://doi.org/10.1016/0047-2727(73)90002-9).

¹⁰ “WHO-IVB-19.10-Eng.Pdf,” accessed January 17, 2020, <https://apps.who.int/iris/bitstream/handle/10665/329389/WHO-IVB-19.10-eng.pdf?ua=1>.

QALYs and that QALYs are not a prescribed measure of benefit in cases where health-related quality of life is not affected by a drug.¹¹ For example, CADTH does not strictly enforce a particular method measurement or a particular Canadian value set for valuing health.¹² INESSS also allows cost-minimization analyses to be performed which will remove QALYs from analyses.

Additionally, it is increasingly recognized that potential benefits may go beyond QALYs and that a QALY gained in the very young or very sick may be valued differently by the Canadian public.¹³ Investment in patented medicines may also raise issues of social justice (adequacy and equity in access); income protection; freedom of choice for consumers; and appropriate autonomy for providers. A synthesis of elements of value is shown in Box 1. Nonetheless, we recognize the focus on QALYs as a good starting point.

Box 1: Examples of Elements of Benefit to Consumers

<p>Health outcomes (population and individual health outcomes)</p> <ul style="list-style-type: none"> • Increased effectiveness • Increased safety <p>Other patient, caregiver and/or population health benefits</p> <ul style="list-style-type: none"> • Reduction of uncertainty (e.g., following diagnosis) • Reduced caregiver burden • Unmet needs • More treatment choice • Improved access to services • Greater equity <p>Health system benefits</p> <ul style="list-style-type: none"> • Decreased net costs of delivery per patient • Lower budget impact • Fewer sunk and other costs (operating costs) • Greater economies of scale or scope • Greater ease of incorporating technology into current system (and ease of future disinvestment) • Improved administration/delivery/supply chain <p>Benefits beyond health system</p> <ul style="list-style-type: none"> • Costs to other areas of government (e.g., education, justice system) • Political acceptability • Social impact (e.g., environmentally friendly) <p>(Adapted from HTAi 2013 Policy Forum Document, Table 1, pg 7)</p>
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¹¹ “Guidelines for the Economic Evaluation of Health Technologies: Canada (4th Edition),” n.d., 76; “Submission_guidance_document.Pdf,” accessed January 17, 2020, https://www.inesss.qc.ca/fileadmin/doc/INESSS/Inscription_medicaments/Fiches_inscription/en/Submission_guidance_document.pdf.

¹² “Guidelines for the Economic Evaluation of Health Technologies: Canada (4th Edition).”

¹³ Chris Skedgel, Allan Wailoo, and Ron Akehurst, “Societal Preferences for Distributive Justice in the Allocation of Health Care Resources: A Latent Class Discrete Choice Experiment,” *Medical Decision Making: An International Journal of the Society for Medical Decision Making* 35, no. 1 (2015): 94–105, <https://doi.org/10.1177/0272989X14547915>.

Market Size

We also agree that the volume effect must be taken into consideration. We do note that new antiviral drugs for chronic hepatitis C infections and age-related macular degeneration contributed to >20% of public drug program¹⁴ spending despite falling well under the PMPRB's proposed threshold for Category 1 drugs.

We have seen similar increases in costs from “non-expensive” drugs. Notably, some of us have observed new drugs and sensors for diabetes contribute to 19.3% year-over-year increases; even larger increases in spending have been seen with drugs for attention-deficit hyperactivity disorder (32.8%). Drugs that fall below the cost-effectiveness threshold but might be considered expensive due to high utilization also include new specialty drugs for migraine headache prophylaxis (\$7,000 per claimant).

2. What aspect(s) of the proposed Guidelines do you disagree with and why?

To this end, while we believe the PMPRB is well-intended in introducing these new measures to regulate excessive pricing, we do think some of the thresholds and approaches may be worthy of reconsideration.

What is affordable?

In the preamble to the PMPRB Draft Guidelines Consultation¹⁵, justification for the new Draft Guidelines is stated as arising from “concern that stronger patent protection for medicines might cause their prices to rise unacceptably and become unaffordable to consumers.” While we agree with the sentiment of this change, we question whether the new Draft Guidelines are fit for purpose in this regard. For example, a drug that costs \$100,000 per year may represent \$20,000 in annual co-payments for an individual with an after-tax income of below \$40,000—unaffordable for an individual wage earner, even with tax concessions. The notion that treatment costs of \$20,000 (or \$100,000) per year could be considered “affordable” in a country with a median after-tax income of individuals not in families of \$52,090 does not make sense.

Similarly, the current approach may determine an excessive price for a new patented medicine that creates large numbers of additional QALYs for an unaffordable price, as is still judged to be fairly priced. These types of therapies may be seen increasingly as the numbers of curative therapies in development continues to rise.¹⁶ In this case, the drug may not be excessively priced but still unaffordable to consumers. Taken together, the price thresholds proposed for

¹⁴ “CIHI : Prescribed Drug Spending in Canada, 2019: A Focus on Public Drug Programs.”

¹⁵ Patented Medicine Prices Review Board Canada, “PMPRB Draft Guidelines Consultation,” aem, January 13, 2020, <https://www.canada.ca/en/patented-medicine-prices-review/services/consultations/draft-guidelines.html#b8>.

¹⁶ Casey Quinn et al., “Estimating the Clinical Pipeline of Cell and Gene Therapies and Their Potential Economic Impact on the US Healthcare System,” *Value in Health: The Journal of the International Society for Pharmacoeconomics and Outcomes Research* 22, no. 6 (2019): 621–26, <https://doi.org/10.1016/j.jval.2019.03.014>.

Category 1 drugs and the fact that a drug may be fairly priced but unaffordable raises questions that the policy intent was achieved. Instead, the measures being proposed seem to be a better recipe for managing overall spend, rather than providing affordable patented medicines to Canadians.

What is expensive?

To determine what new drugs might be excessively priced, the new Draft Guidelines propose a threshold of half of GDP per capita over a 12-month period as a screening criterion. Why this is so is not clear. While (Q19 of) the backgrounder offers the answer to “Why” as “stipulated in section 4.1(5) of the Amended Regulations.”, no justification is provided. At the current Canadian GDP per capita, this annual cost is roughly \$30,000. This is three times the value that private plan sponsors use (\$10,000) as an informal threshold for expensive drugs. With an increasing number of high-volume drugs under \$10,000, plan sponsors are also increasingly questioning whether this value should actually be lower. A recent survey of health benefit plans revealed only 41% of plan sponsors were “confident that a drug costing \$24,000 annually would be covered”.¹⁷ We believe what is defined as expensive should align more closely with a consideration of the means of those who must pay for drugs.

International Price Referencing

While we recognize many countries with modern healthcare systems have adopted international reference-based pricing (see Table 1), we are concerned that this approach falls short of considering the value of a patented medicine to consumers within a Canadian context. That is, the price of a newly patented medicine, such as new patented medicine, will be determined by what drugs are already available in those jurisdictions and this will vary among the newly proposed PMPRB11.

International reference pricing also creates conditions where prices are referenced to countries with less ability to pay (or less activity related to the pharmaceutical sector), unfairly affecting producers. Conversely, externally referenced prices in jurisdictions with more pharmaceutical sector-related economic activity (and, therefore, a greater ability to pay) may be insufficiently rewarding consumers, as higher prices result will lead to lower consumer use.

¹⁷ “THE SANOFI CANADA HEALTHCARE SURVEY, 2018,” n.d., 36.

Table 1: Price regulation schemes and their application internationally

Scheme	Jurisdiction(s) , e.g.
Free pricing – Prices are not subject to regulation; producer charges what market will bear.	USA
Cost-based pricing – Prices are based on marginal costs of production (costs of research, production, promotion, and distribution).	India (prior to 2013)
Reference pricing – Prices are set through comparison to an existing standard. An <i>internal</i> reference price generally involves comparing the price of the drug to another drug with similar chemical, pharmacologic, or therapeutic properties. ¹⁸ An <i>external</i> reference price involves comparing the price of a drug to prices in other jurisdictions selected according to some notion of comparability, usually economic or geographic. ¹⁹	Most European Countries (not UK, Cyprus, Malta, Bulgaria)
Profit-based pricing – Prices may be set freely but rebates paid to regulator based on profits.	UK
Value-based pricing – Prices are determined based on perceived or realized benefits; nominally a pre-determined balance of benefits to both consumers and producers.	Germany, Sweden

Two-tiered pricing

We also question the two-tiered pricing structure of maximum rebated and maximum list prices being proposed. Our plan members will still be subject to maximum list prices for which insurers may or may not receive rebates and who have no obligation to pass on the savings. As everyone ultimately pays for the health of others, we question the logic of not allowing value-based prices for all consumers. Two-tiered regulations for pricing also raises the concern that companies will simply stop offering rebates altogether, as a rational response to price reductions and in order to preserve their bottom line. This will ultimately raise the prices of medicine for everyone. In sum, we do not feel that the uninsured public or our plan members are more “willing to pay” higher medication prices for medications than the general public, as the guidelines imply.

Costs of Production

Fairness in price must not only consider what consumers are willing to give up to get something (marginal utility), but also what suppliers are willing to give up (marginal costs). While the current guidelines focus on the QALY as a unit of additional benefit, they do not consider the costs borne by manufacturers. While we recognize marginal costs of production may be difficult to characterize or for

¹⁸ Lopez-Casasnovas and Puig-Junoy. Review of the Literature on Reference Pricing

¹⁹ Organisation for Economic Co-operation and Development. *Pharmaceutical Pricing Policies in a Global Market*

patentees to report, the current approach implies that the costs of drug production is the same for all drugs, despite much evidence to the contrary²⁰. This could mean some suppliers are unfairly penalized, if a drug is expensive to develop. However, it also means consumers may be unfairly penalized, if a drug with significant healthcare benefits is developed at little cost. Regulations that attempt to simulate market conditions should consider the fact that goods with lower marginal costs of production would deserve lower prices (than other goods producing the same benefits that cost more to produce). We would encourage the PMPRB to see how this could be factored into the price calculations.

Category 1 Drugs

While we are happy the new guidelines use an incremental cost per quality-adjusted life-year (QALY) threshold as a means of comparing prices with their proposed benefits, we question the thresholds used. In establishing this threshold, the PMPRB cites empirical work that concluded a \$30,000 cost per QALY threshold, and that this is “broadly in line with empirical estimates of supply-side thresholds in other jurisdictions with similar wealth and pharmaceutical market characteristics as Canada”.

Given the evidence presented of a clear line that characterizes when a patented medicines costs exceed its benefits, we question the ultimate value of \$60,000/QALY ultimately proposed. The justification for doubling of what seems to be a reasonable measure of opportunity cost provided is “anecdotal evidence” and “the relatively embryonic state of empirical work”. This doubling of the empirically measured threshold implies an almost doubling of prices. We feel the PMPRB may have been incautious in using “anecdotal evidence” to trump the evidence base and question why they only meet the needs of Canadians halfway. In short, we believe the threshold should be in line with measured evidence. Prices based on a \$60,000 / QALY are still excessive by any measure, and there are no market forces within the current system that are likely to lower them further.

Similarly, we also question the doubling of empirically measured market size thresholds as provided in section (11) of the Backgrounder.²¹ This appears to be based on a concern about the volume of PMPRB reviews rather than any empirical notions of excessiveness. It is our belief that if PMPRB is concerned about the staffing / human resources required to regulate a larger basket of drugs, then we would prefer to see more resources dedicated to the PMPRB, to enforce evidence-based thresholds rather than a weaker approach.

²⁰ Vinay Prasad and Sham Mailankody, “Research and Development Spending to Bring a Single Cancer Drug to Market and Revenues After Approval,” *JAMA Internal Medicine* 177, no. 11 (November 1, 2017): 1569–75, <https://doi.org/10.1001/jamainternmed.2017.3601>.

²¹ Canada, “PMPRB Draft Guidelines Consultation.”

The fallacy of Canadians valuing rare

The Draft Guidelines also provide substantial maximum price increases for drugs with “very low prevalence”. While we recognize an attempt to recognize those with *debilitating* rare disorders, we feel the guidelines fall short on two fronts:

- First, there is abundant evidence from Canada and abroad that it is not the prevalence of a disease that is valued by Canadians *per se*, but rather age and measures of health-related quality.²² While the original intent of providing premiums for rare conditions was these rare diseases, such as congenital illnesses, are typically those with excessive mortality and morbidity, this is not always the case. In an era of therapies that increasingly rely on biomarkers and genomic testing, common diseases can be made to be “rare”.²³ As such, we believe the sole focus on prevalence is unwarranted and requires qualification to reflect available empirical evidence. It also reflects similar findings among care providers and insurers internationally.²⁴
- Lastly, while we recognize the threshold for rare is subjective, it seems to be out of line with international jurisdictions, with a global average of 1/2500, which is also used in the UK.²⁵ Australia, one of the proposed basket countries in your example, uses a threshold of 9/100,000 for rare.²⁶ Given the use of the new PMPRB11, one approach may be to take a population-weighted average of thresholds for rare in these countries. Although as per our previous comments, this should also be qualified by traits of rarity (i.e., age, and excessive morbidity / mortality) and that these have a basis in evidence.

²² Skedgel, Wailoo, and Akehurst, “Societal Preferences for Distributive Justice in the Allocation of Health Care Resources”; Arna S. Desser et al., “Societal Views on Orphan Drugs: Cross Sectional Survey of Norwegians Aged 40 to 67,” *BMJ (Clinical Research Ed.)* 341 (September 22, 2010): c4715, <https://doi.org/10.1136/bmj.c4715>; Siobhan M. Bourke, Catrin O. Plumpton, and Dyfrig A. Hughes, “Societal Preferences for Funding Orphan Drugs in the United Kingdom: An Application of Person Trade-Off and Discrete Choice Experiment Methods,” *Value in Health: The Journal of the International Society for Pharmacoeconomics and Outcomes Research* 21, no. 5 (2018): 538–46, <https://doi.org/10.1016/j.jval.2017.12.026>.

²³ Ameet Sarpatwari and Aaron S. Kesselheim, “Reforming the Orphan Drug Act for the 21st Century,” *New England Journal of Medicine* 381, no. 2 (July 11, 2019): 106–8, <https://doi.org/10.1056/NEJMp1902943>.

²⁴ Goran Medic et al., “Do Payers Value Rarity? An Analysis of the Relationship between Disease Rarity and Orphan Drug Prices in Europe,” *Journal of Market Access & Health Policy* 5, no. 1 (April 10, 2017), <https://doi.org/10.1080/20016689.2017.1299665>; Arna S. Desser, “Prioritizing Treatment of Rare Diseases: A Survey of Preferences of Norwegian Doctors,” *Social Science & Medicine (1982)* 94 (October 2013): 56–62, <https://doi.org/10.1016/j.socscimed.2013.06.019>; Desser et al., “Societal Views on Orphan Drugs”; Bourke, Plumpton, and Hughes, “Societal Preferences for Funding Orphan Drugs in the United Kingdom.”

²⁵ Trevor Richter et al., “Rare Disease Terminology and Definitions—A Systematic Global Review: Report of the ISPOR Rare Disease Special Interest Group,” *Value in Health: The Journal of the International Society for Pharmacoeconomics and Outcomes Research* 18, no. 6 (September 2015): 906–14, <https://doi.org/10.1016/j.jval.2015.05.008>.

²⁶ Richter et al.

3. What impact do you hope these reforms will achieve for you, your organization and Canadian patients?

We are cautiously optimistic the current Draft Guidelines, as applied, will lower costs of patented medicines for both our plan members and insurance providers, providing additional opportunities for health and medicines coverage for members. However, we are aware that regulation of prices can have untoward effects and there are no one-size-fits all solutions. As per our previous comments, we are also concerned that expensive drugs may still be unaffordable to some, that definitions of expensive may be out of step with payers, and that the guidelines will fall short of achieving reductions in expenditures that are in line with our current budgetary constraints.

4. If you had to design a price test using the new factors to ensure Canadian prices are fair and affordable, what price test would you suggest?

Consistent with our previous comments, we do believe some factors may need to be accounted for, particularly factors beyond prevalence (i.e., age and morbidity) for those with rare conditions, and marginal costs of production for suppliers. We also fail to recognize the value of using international reference pricing or two-tiered pricing approaches when proposing Guidelines intended to promote *affordable* prices for *all Canadians*.

However, our comments are more broadly focused on the choices of thresholds for the measures proposed. Namely, we are concerned that the ICER threshold of \$60,000 / QALY may be too high given empirical evidence, and that one-half GDP per capita annually as a means of identifying an expensive therapy is not consistent with what is affordable. Similarly, some thresholds were chosen based on the volume of drugs that would be captured, rather than an alignment with the policy intent (fair pricing for consumers). While we recognize an attempt to be practical, we question changing problems to fit solutions.

Concluding Remarks

We appreciate this opportunity to submit written consultation to the proposed Guidelines and hope this written feedback is helpful.

In summary, while we agree with the intent of the proposed Guidelines, we believe they fall short of their stated aim. Simply put, the intent of any policy with the goal of affordable prices for all Canadians must lead to *affordable* prices for *all Canadians*. The proposed Guidelines in their current form do not go far enough. While we object to the various thresholds proposed as they do not align with current evidence, we do not object to the measures per se. We hope the PMPRB also considers the real constraints of private plan sponsors, and the need to help our members, and all Canadians.