October 2016

Submission to the Patented Medicines Pricing Review Board (PMRPB)

Table of Contents

Signatories

As a group of concerned patient organizations, we have carefully considered the Discussion Paper, and are pleased with the PMPRB's openness to engage with patients on this review. We are looking forward to the opportunity to develop – with the PMPRB – a range of options for improving the operation of Canada's federal price review body for the benefit of Canadian patients.

Sincerely,

CANADIAN CANCER SURVIVOR NETWORK

JACKIE MANTHORNE
President & CEO

LOUISE BINDER
Health Policy Consultant

Canadian Psoriasis Network / Réseau canadien du psoriasis

SIMMIE SMITH
President

BRENDA SPINOZZI
Vice President

CANADIAN PKU AND ALLIED DISORDERS

JOHN ADAMS
President & CEO

CNETS CANADA — Carcinoid-NeuroEndocrine TUMOUR SOCIETY CANADA

JACKIE HERMAN
President

SCHIZOPHRENIA SOCIETY OF ONTARIO
A REASON TO HOPE

MARY ALBERTI
Chief Executive Officer

save your skin FOUNDATION / LA FONDATION sauve ta peau

SABRINA HANNA
Executive Director

BETTER CARE FASTER
A Campaign for Ontario

MYÉLOME CANADA
MISSION : MAÎTRISER LE MYÉLOME
MYELOMA CANADA
MAKING MYELOMA MATTER

MARTINE ELIAS
Director – Access, Advocacy and Community Relations

Introduction and Executive Summary

John- Peter Bradford

CEO

The signatories to this submission laud the PMPRB for its foresight and vision in developing an audacious and far-reaching Discussion Paper outlining a potential new direction for the organization. The Patented Medicine Prices Review Board (PMPRB) has clearly recognized the evolution in the health policy landscape since its inception including the area of drug pricing.

Clearly PMPRB has recognized the changes in the health policy landscape since its inception, including in the area of drug pricing. New government processes have recently been created including the pan-Canadian Pharmaceutical Alliance (pCPA) and existing health technology agencies including the Canadian Agency for Drugs and Technologies in Health (CADTH) and its counterpart in Quebec, L'Institut national d'excellence en santé et en services sociaux (INESSS) are evolving their roles and policies as well. With the development of many more innovative medicines and health technologies and the rapidly expanding research in such disease areas as oncology, rare diseases and mental illness sustainability and cost pressures are increasing in both the public and private health care environment. This has already begun to limit access to treatments and has the potential to severely limit access to badly needed medicines for life threatening and serious debilitating illnesses and disabilities unless properly managed and unless all stakeholders examine their present mandate, policies and practices and re-calibrate accordingly.

We continue to believe that the role of PMPRB is to monitor the price of patented medicines, to ensure they do not exceed a ceiling, and that this must remain its primary *raison d'etre*. We see no reason to turn this essential role over to another agency when it has the experience and infrastructure to do this.

It is most important to ensure that we look to the future of research and development and not to the past when we consider all of these factors. In many instances, future research is not going to be large multi-centre trials and

personalized medicine is becoming more prevalent. Thus, the way we measure therapeutic improvement and efficacy is going to have to change also. All health systems, including are going to have to revisit how they measure these factors within their jurisdiction.

In this context, our submission responds to a number of questions and considerations, beginning with the role of the PMPRB. Highlights of our recommendations are set out, below.

- The *Patent Act and Regulations* set out the legal framework for PMPRB's work which is to determine whether the proposed sale or ceiling price of a drug is excessive. The legal framework provides some guidance on what to consider in terms of excessive pricing. Thus, a definition of excessive that is based on factors other than those that set a ceiling point is irrelevant in the present health policy environment. Definitions that include any of: "value," " affordability," "sustainability," "ability to pay," or similar concepts are already within the jurisdiction of other drug policy agencies across the country.
- We support the consumer protection mandate of PMPRB and see no reason for PMPRB to expand its consumer protection mandate to private insurers. Private industry is structured to reap the rewards and to deal with the issues that arise within its jurisdiction and it should continue to do so. It has the power to counterbalance the monopoly power of the pharmaceutical industry, where such monopoly exists, that does not exist in the same way in the public system.
- PMPRB also plays an important role in drug price monitoring for the generic side.
- International comparators should be reviewed by PMPRB after the initial determination phase and as a second review only. If it finds the price it has pegged as a non-excessive ceiling price is far out of alignment with other relevant international countries, then it should determine why that is and consider realignment if appropriate.
- In analyzing an excessive ceiling price, the deliberation must include an analysis of therapeutic benefit measured by scientific evidence and patient

input based on real world experience, comparable (e.g., with equivalent safety, efficacy, management profile) competitor product prices, comparative prices for drugs in other disease areas in similar circumstances (e.g., the price of a cure for a life threatening disease in one therapeutic area extrapolated to another area) and to a lesser extent international pricing in comparable countries.

- Where no comparable product exists against which to measure the price, which may well mean an important therapeutic benefit, there may be a heightened need for consumer protection, since the manufacturer has greater leverage to use this monopoly advantage to the disadvantage of public payers or individuals purchasing the drug. Thus, we propose the creation of a dispute resolution mechanism to monitor these situations. No such mechanism would be required for other categories of drugs that PMPRB reviews and in fact perhaps less scrutiny is required in those areas than is undertaken presently, permitting redeployment of those resources to the minority of cases in this "monopoly" position.
- The Consumer Price Index (CPI) is not relevant and should be removed from the *Compendium of Policies, Guidelines and Procedures* (Guidelines). In the normal course of events, prices should generally drop over time, not be permitted to rise automatically.
- One question for future consideration is the extent to which PMPRB should take into account the cost of companion diagnostics paid for by companies in determining an excessive price.
- Currently the profits that are clawed back from excessive pricing situations go back into general federal coffers rather than be disseminated to public and private payers that have paid these higher prices minus any costs that PMPRB has incurred to win its case. This should be reconsidered.
- As consumers we think it is time to reconsider the legal rules which prevent or inhibit improvements in patient participation, transparency and public accountability of PMPRB, such as the restrictions on public disclosure of the reasons for decisions on whether or not a patent medicine is categorized as a "breakthrough" and the absence of any opportunity for patient groups to make submissions on how a patent medicine should be categorized for maximum pricing purposes.

Role of PMPRB

This is the perfect opportunity to ask whether PMPRB still has a discrete role in the drug pricing landscape in Canada, and if so, has it changed given the environmental changes described above.

If it does still have a role, the next question is whether the mandate and laws, regulations, guidelines and practices that support it require realignment.

Originally, the PMPRB was created to ensure that pharmaceutical companies that were given patent protection, a virtual monopoly for a period of time to sell their products without generic treatment competition, met their compensatory obligations to keep pricing from being "excessive."

It also has had a reporting mandate for drug trends. Over time, its mandate expanded to include monitoring of generic drug prices for governments. It also monitors Research and Development in Canada.

Based on a recent Supreme Court of Canada decision, and PMPRB's analysis of the lack of success it perceives it has had in keeping drug prices low (i.e., non-excessive) and investment up (a role that has been beyond its power to control from the outset), it now posits that its role is the gatekeeper to avoid the brand name drug companies from using their monopoly status to the detriment of consumers. It is defined as being a consumer protection agency in its mandate and it wishes to align its processes to match this role.

As mentioned above, many agencies have evolved and others have expanded their roles since PMPRB's inception. This begs the question as to whether these agencies have taken over the role that PMPRB had or now wishes to have.

Certainly CADTH and pCPA have an impact on the price the public system pays for drugs based on what that market will bear (i.e., affordability). They do not, however, determine the price that starts the bidding or the ceiling price based on factors that determine if the price is excessive for Canada. If PMPRB were not in existence how would that opening price be determined? Probably by the pharmaceutical company making a first offer to pCPA. On what would it base this offer? No doubt the size of the potential market opportunity would be relevant

and whether there are other entrants into the market as well. Pricing set in other countries that they consider comparable would also probably be considered.

An objective analysis of factors to determine a ceiling for the starting ask or price, would still have to be done. As such pCPA would have to do much of the same analysis that PMPRB does now, with either the same or potentially different criteria and benchmarks. Thus, we conclude that what PMPRB is doing on the brand or patented medicine side is necessary. We see no reason to turn this role over to another agency when it has the experience and infrastructure to do this, although this submission does discuss whether it needs to do so differently. We do caution the PMPRB, however, to ensure that it stays within its jurisdiction as set out in *Patent Act* and *Regulations* and not to stray outside of its role of monitoring "excessive " pricing and into the questions of "value" or "affordability" that, we submit, are already within the jurisdiction of other agencies including CADTH, pCPA and public drug budgets.

We see no reason for PMPRB to expand its consumer protection mandate to private insurers either. Private industry is structured to reap the rewards and to deal with the issues that arise within its jurisdiction and it should continue to do so. It has the power to counterbalance the monopoly power of the pharmaceutical industry that does not exist in the same way in the public system.

PMPRB also plays an important role in drug price monitoring for the generic side.

Question 1 – What Does "Excessive" Mean?

From a practical personal perspective, patients requiring a drug look at the drug through the lens of access. Thus, any price that ultimately, directly or indirectly, is responsible for a drug being deemed beyond a purchaser's budget, although safe, effective and needed for patient continuation of life and significant enhancement of quality of life is colloquially excessive for patients. (Most thoughtful patients will concede that drugs for non-life threatening and minor ailments that create a drain on opportunity costs for other drugs that are necessary medications do not have the same impact as other drugs.)

Of course each of the other stakeholders will have its own subjective definition for "excessive." This is why an objective, transparent, reasonable definition needs

to be developed. This is, however, not a definition of “value” or of “affordability” which are also relevant to buying power but outside PMPRB’s jurisdiction, resting with CADTH, INESSS and pCPA.

In fact, what any stakeholder would describe as excessive is not relevant to PMPRB except in the context of its mandate. The real question in the current environment is not what does excessive mean but rather what factors should we consider in our Guidelines to determine excessive prices given the mandatory requirements in the *Patent Act* and *Regulations*, determined by Parliament, not by PMPRB.

One of the ongoing pressure points with drug pricing is that drugs are manufactured and supplied by private industry in the same manner as other consumer products (e.g., jeans, wine, desks, etc.) but there is an undeniable qualitative difference in that without many drugs we may die or live a very poor quality of life. Thus, they are more like food, water and shelter than other consumer goods. If we cannot afford them, it is not just an inconvenience in many cases but a matter of length and/or quality of life.

To some extent we recognize this difference in that we have created a public system for making drugs accessible to the extent that our predetermined health budgets will allow. Generally the intention is that basic necessities for drugs are covered for eligible recipients (e.g., those least likely to be able to afford them in any other way).

For purposes of this consultation, individual subjective definitions of “excessive“ are not relevant. The definition of “excessive” in this context must be dictated primarily by the jurisdiction of the PMPRB as set out legally in the *Patent Act and Regulations* and relevant court decisions. Section 85(1) of the *Act* is explicit about the factors that are to be taken into account in determining an excessive price.

The size of the population it treats and the impact on the drug budget are also not within the domain of PMPRB. Indeed pCPA and provincial/territorial jurisdictions will decide these in the public domain and insurers will decide them for the private sector in consultation with plan sponsors.

Other important economic considerations are the impact on other silos of the health budget and the impact on industry by lowering costs to the private sector

directly or indirectly that in turn, impact GDP. These should be taken into account at the CADTH, pCPA levels but often are not.

If the PMPRB is intended to elicit a definition of excessive that is based on factors other than those that set a ceiling point, we submit that the question is irrelevant in the present health policy environment. Definitions that include any of: “value,” “affordability,” “sustainability,” “ability to pay” or similar concepts are already within the jurisdiction of other drug policy agencies across the country.

Question 2 – Role of International Comparators

We should look at relevant or comparable international comparators but only as a secondary check and balance after the other factors set out above have been considered.

Relevance includes:

- demographics
- political structure
- economics
- health systems structure
- industrial base
- research and development
- value systems in health (i.e., are we willing to pay more so that poorer countries can have access to these drugs)

The United States does not fit these criteria because of factors including its size and health care structures. It is, however, our neighbour and has a huge impact on our economy. Before we decide whether or not to discard it, we must determine the unintended consequences of doing so.

We also have the problem of the fluctuation of the Canadian dollar and how to manage this variable. Our present approach does not sufficiently take into account sudden and significant downturns in our economy that have an

immediate impact on our dollar. We need to find a way to do so if we are to compare to international prices.

If PMPRB reviews international comparators after the initial phase and finds the price it has pegged as a non-excessive ceiling price is far out of alignment with other relevant international countries, then it should determine why that is and consider realignment if appropriate.

Question 3 – Guidelines Section 85

Role of Therapeutic Benefit

In analyzing an excessive ceiling price, we submit that the deliberation must include an analysis of therapeutic benefit measured by scientific evidence patient input, comparable (e.g., with equivalent safety, efficacy, management profile), competitor product prices, comparative prices for drugs in other disease areas in similar circumstances (e.g., the price of a cure for a life threatening disease in one therapeutic area extrapolated to another area) and to a lesser extent international pricing in comparable countries.

Where no comparable product exists against which to measure the price, which may well mean an important therapeutic benefit, there is certainly a heightened need for the consumer protection concern since the manufacturer has greater leverage to use this monopoly advantage to the disadvantage of public payers or individuals purchasing the drug. Thus, we propose the creation of a dispute resolution mechanism to monitor these situations. No such mechanism is required for other categories of drugs that PMPRB reviews. In fact less scrutiny is required in those areas than presently is undertaken, permitting redeployment of those resources to the minority of cases in this "monopoly" position. For example, in the area of "me too" drugs, where most of the "excessive" pricing issues arise at PMPRB, perhaps only extremely "excessive" pricing would be investigated proactively by PMPRB (e.g., prices more than 25% of prices for other drugs in the same class, and other investigations could be handled based on a complaint-initiated process).

The Consumer Price Index is not relevant and should be removed from the Guidelines. In the normal course of events, prices should generally drop over time, not be permitted to rise automatically.

One question for future consideration is the extent to which PMPRB should take into account the cost of companion diagnostics paid for by companies in determining an excessive price.

Question 4 – Range placement

Breakthrough based on the international median is probably acceptable if the right countries are in the basket and depending on how those countries set their price. We should follow what other countries do regarding the use of median prices unless there is good reason not to do so.

Question 5 – Impact of Research and Development

Research and Development investments to meet the PMPRB requirements are not relevant anymore. In the globalized research ecosystem of the pharmaceutical companies, PMPRB has less and less leverage to impact this and it is clearly not being determined by patent protection. A more relevant approach today would be to suggest investments in more than the typical Phase I-IV clinical trials. For example, epidemiological research, including real world evidence evaluations that are geared to address Canadian health policy issues should be considered and encouraged.

Federal and provincial/territorial innovation, industry and economic development ministers must determine how to incentivize companies to do business in Canada.

In addition, Canada should consider a formal *Orphan Drugs Act* like many other countries have, to encourage research and development for rare diseases in Canada.

Question 6 – Categorization

Therapeutic benefit cannot be ignored in a private market system. Recognizing work that increases health with profits is built into the system to encourage innovation. That being said, if something is good and a lot of people want it, the price should reflect increased market share and volume and thus should decrease over time.

In determining therapeutic benefit one should take into account patient information as part of the scientific review.

Also in determining the level of therapeutic benefit, route of administration in terms of the impact on willingness to take a treatment (e.g., needles vs. pills and many pills/number of times a day) and ability to be compliant to the dosage regimen should be given a higher weight than the other secondary factors.

An important consideration for the future is what we are going to do once factors including immuno-oncology and targeted therapies lead to personalized medicine and the kinds of trial data we have had in the past are no longer available or relevant.

PMPRB has expressed its concerns that if it does not deal with “monopoly” profit situations rather than just “normal” profits it is not doing its job of protecting Canadians from excessive pricing. We agree. This situation generally arises in the area of “breakthrough” drugs. This category does not make up a huge portion of drugs that come into Canada. We should not be creating a more complex price monitoring system than we need to deal with outlier situations. This merely discourages research and development and introduction of new drugs into the Canadian market.

As proposed above, PMPRB should consider the introduction of an expeditious alternate dispute resolution mechanism for such situations rather than developing complex guidelines that have the proverbial tail wagging the dog. This would still leave the legally binding hearing alternative if that additional process is unsuccessful.

In addition, if the PMPRB and other stakeholders need a discreet pathway for “rare” or “orphan” drugs they should work with Health Canada that has

developed a rare disease framework and other regulatory pathways to develop such a pathway.

Question 7 – Levels of Oversight

As discussed above, different levels of oversight are fine. We caution, however, to be considerate about the definition of "me too" drugs in this context. Reliance should be placed heavily on real world experience in this regard.

Question 8 – Ceiling Price Revisions

Price ceilings should certainly not be allowed to rise over time. It is worth discussing what benefit there is in revising the ceiling price over time. The value of so doing is not immediately obvious since other pricing systems will have taken over after the initial ceiling price is set.

This review may have some relevance in the context of indication creep.

Question 9 – Private and Public Sector

This question is irrelevant and subjective. The two processes are used in the context of entirely different markets and purposes so just because they are different does not mean they are discriminatory. Let private industry solve its own problems unless it is willing to share its high profits with the public system.

Question 10 – Other Aspects

None at present.

Question 11 – Prospective versus Retrospective

We cannot answer this until we know the changes that are being planned but generally any substantive changes should be prospective only as it is generally unfair to change the rules of engagement retrospectively when stakeholders have relied on them.

Question 12 – Other issues

We wonder why the profits that are clawed back from excessive pricing situations go back into general federal coffers rather than be disseminated to public and private payers that have paid these higher prices minus any costs that PMPRB has incurred to win its case?