Network of Rare Blood Disorder Organizations
Réseau des Associations Vouées aux Troubles Sanguins Rares

October 21, 2016

Patented Medicine Prices Review Board (PMPRB)
(Rethinking the Guidelines)
PMPRB.Consultations.CEPMB@pmprb-cepmb.gc.ca

Dear PMPRB Members,

On behalf of the Network of Rare Blood Disorder Organizations (NRBDO) we are writing today to submit our feedback to the PMPRB Guidelines Modernization: Discussion Paper consultation. The NRBDO is a pan-Canadian coalition of not-for-profit organizations representing people with rare blood disorders and/or people with a chronic condition who are recipients of blood or blood products or their alternatives.

We welcome this opportunity to provide feedback on the Guidelines Modernization Discussion Paper on behalf of patients. Considering the fact that desired Research and Development R&D commitments are not being met and the changes in the market and regulatory environment since the 1980s, the following areas of the patent and pricing guidelines stood out as needing adjustment and review in order to enable the PMPRB to ensure that Canadians have access to patented medicines at affordable prices.

The Mandate of the PMPRB

We acknowledge and affirm the importance of the mandate of the PMPRB, having the two roles as identified on the PMPRB website:

> Regulatory – To ensure that prices charged by patentees for patented medicines sold in Canada are not excessive;
>
> Reporting – To report on pharmaceutical trends of all medicines and on R&D spending by pharmaceutical patentees.

The purpose of the PMPRB is to **protect consumers, not reward innovation**. We recognize that the original creation of the PMPRB intertwined pricing with a commitment to industry R&D levels. At this point we believe that needs to be re-assessed as the pricing method has not resulted in the desired R&D commitments being met.

The **PMPRB needs to recalibrate to provide a strong counter-balance to the measures put in place to reward pharmaceutical companies via the Patent Act, rather than trying to take a balanced approach itself**. The expertise and experience that the PMPRB has developed over the years is valuable and should be leveraged.

Therapeutic Benefit

With therapeutic benefit of medicines being considered in the pricing, the PMPRB is duplicating the work of CADTH and INESSS. We believe that the **reward for innovation should be market exclusivity, not premium pricing.**

The NRBDO members have been beneficiaries of numerous niche-busting drugs in recent years. These include drugs to treat diseases that had little or no effective treatment before, such as Vidaza and Revlimid to treat myelodysplastic syndrome (MDS), and Soliris to treat paroxysmal nocturnal hemoglobinuria (PNH). As well there have been significant improvements in therapeutic options, sometimes dramatically changing the landscape of what is available, such as Exjade, an oral iron chelator. While these drugs are welcome life-saving or significant life-extending medications, they are much too expensive for any individual to afford. In fact, sometimes even the insurance co-payment is unaffordable. Thankfully these medications are on many, if not all, provincial formularies, however, some provinces have placed restrictions on access to these medications. These vary from payer to payer and are not disclosed, but we believe this is a cost-saving measure, to the detriment of patients. While affordability is an issue, the issue of more concern to us is when access is restricted due to pricing.

In essence what is most important to us is that patients have access to the best possible drugs at the best possible price.

Finally, from a patient point of view, it would be a greater benefit to create an environment that helps support the creation and access to clinical trials as opposed to increased R&D.

International Pricing Comparisons

The countries that comprise the PMPRB7 were selected because they modeled pharmaceutical R&D levels sought in Canada. Several of these countries consistently pay among the highest prices in the world for drugs. Using these countries as comparators has led to Canadians routinely paying among the highest patented drug prices in the world, yet Canada's R&D levels have not increased as promised.

We feel that a different set of international comparator countries are needed. While it is not clear to us what the appropriate "basket of countries" is, we have a few observations. In particular, **we consider the US to be an outlier country that we should not be compared to**. When conducting an international examination of drug prices, Canada should consider a selection of countries that are similar to Canada in factors such as in economics, demographics, social and political structures, health care system and/or population health goals.

One question we ask is: What international best practices can Canada learn from? Since other countries have to face similar challenges for paying for pharmaceutical drugs within a public payer plan (or even

within an insurance plan), what practices have been helpful to ensure drug availability to patients, and cost containment? What can be learned from these other countries?

Although it is outside the scope of the PMPRB, the fragmentation and duplication in the Canadian federal/provincial/territorial funding system creates variations across the country, delays in government funding of new treatments, an absence of certain treatment options, and a host of other problems for patients who are awaiting a new medication. The national buying power of other nations may be a reason for better pricing of - and earlier access to - new medications.

Lack of Pricing Transparency

Of great concern to us is the lack of transparency regarding prices actually charged by the pharmaceutical industry, which leads to benchmarking at artificial levels, both internationally and based on past drugs.

The PMPRB must be granted insight into "secret" prices being established by the pharmaceutical industry, or stop benchmarking based on "list" prices, using either medians, minimum prices, or estimated discounted prices.

Orphan Indications vs. other indications – the ability to "re-bench"

Often new drugs are introduced as an orphan drug and secure premium pricing, and then new indications are later added which broaden the market after the high price is established. One example that affects our members is Soliris. While PNH was the first indication for Soliris, it has now expanded to include Atypical Hemolytic Uremic Syndrome (aHUS) as well, with possible new indications on the horizon.

Of particular interest to patient groups serving those with rare disorders, is the need for the PMPRB to **have – and exercise – the ability to re-bench drugs periodically, and when market conditions change, such as new indications and/or changes to CADTH/INESSS recommendations.** The total market should be considered when pricing a drug, not just a sole indication.

Addressing these factors will contribute to an improved drug pricing system that monitors and regulates drug prices keeping patients and population health top of mind.

Reporting

The reporting role of the PMPRB is important to patients, providing valuable information. The public process for hearings is also of benefit to patients, and we hope to see these continue.

We are encouraged by the PMPRB's level of engagement and openness in this process. Your mandate is an important one, and the revision of your guidelines will contribute to a strengthening of our country's ability to effectively enable access to medicines while protecting Canadians from over-pricing.

We appreciate this opportunity to submit our thoughts to this important consultation on behalf of patients living with rare blood disorders. The NRBDO includes representation from the following member groups:

- Answering TTP (Thrombotic Thrombocytopenic Purpura)
- Aplastic Anemia and Myelodysplasia Association of Canada (AAMAC)
- Canadian Association for Porphyria (CAP)
- Canadian Hemophilia Society (CHS)
- Canadian Immunodeficiencies Patient Organization (CIPO)
- Canadian Organization for Rare Disorders (CORD)
- Fanconi Canada
- HAE Canada (Hereditary Angioedema)
- HHT Canada THH
- Sickle Cell Disease Association of Canada (SCDAC)
- Thalassemia Foundation of Canada (TCF)

Sincerely,

On behalf of the NRBDO,
Jennifer van Gennip, Administrative Coordinator
Silvia Marchesin, Board Member