CANADIAN ASSOCIATION OF PNH PATIENTS

July 29, 2020

The Canadian Association of PNH Patients respectfully submits the following in response to the consultation currently underway regarding proposed amendments to the Regulations governing the Patented Medicine Prices Review Board (PMPRB). With respect to the changes made to the November 2019 Draft Guidelines, and as reflected in the June 2020 Draft Guidelines, we remain concerned that the health of Canadians living with paroxysmal nocturnal hemoglobinuria (PNH) continues to be put at risk by the uncertainty generated by the PMPRB reform process to date. **Without treatment, this life-threatening blood disorder has a death rate of more than 40% after five years; as such, we cannot accept any additional and unnecessary risks to our lives.**

As a PNH patient myself, I have faced uncertainty numerous times since I was diagnosed with this very frightening disease, but thankfully, at every turn, I was able to get access to the care and treatment I needed to go on living, and there are many others like me across Canada. If I had not been able to access the only treatment available for PNH – first through compassionate access from the manufacturer and then through public funding – I would not be alive today. And while it is costly, this medication **saves patients from certain death and returns their life expectancy to that of a normal healthy person** of the same age and sex (*Blood Journal, 2011*), but it is still the **only one accessible to PNH patients in Canada**. Like any other disease, one treatment will not be the solution for everyone, so options are needed – especially with a life-threatening disease like PNH. As such, we view any new or existing barriers to treatment as unacceptable to our patient community, and the uncertainty caused by the proposed PMPRB reforms continues to be the barrier of greatest concern.

As a result of the pharmaceutical industry's reinvestment in research, enabled by both public and private drug funding, the science is getting better and the number of targeted, specialty medicines is consistently increasing. Many of those treatments now being discovered are for rare diseases, and where drug pricing regulations and legislation exist to support reimbursement, lives are being improved, extended and saved. **At last count, there were 18 molecules being studied in clinical trials around the world for the treatment of PNH. This is unprecedented, but largely meaningless to Canadian patients as only one of these clinical trials has sites in Canada.** None of the companies conducting these trials – even those in Phase 3 – have confirmed that Canada is included in their global launch plans. Given the uncertainty caused by the proposed PMPRB reforms, we are not surprised, but we are extremely concerned for what the future holds.

In one notable case, where the manufacturer took a chance and sought approval for a new PNH medication in Canada, uncertainty in this market has led to a halt in the reimbursement process, rendering it inaccessible to those who depend on public funding. This treatment, a reformulation of the only existing drug for PNH, brings a very timely advance in delivery by reducing the frequency of infusions from every two weeks to every eight weeks. **Eliminating three of four visits to a clinic or hospital-based infusion centre goes a long way to keeping immune-compromised PNH patients safe during the global COVID-19 pandemic, but not here**

in Canada. Elsewhere in the world, up to 70% of PNH patients have been able to switch to this new treatment, but due to the uncertainty caused by the proposed PMPRB reforms, Canadian patients are still awaiting access – and needlessly being exposed to health risks from COVID-19. We also understand that the countries making this reformulated treatment option accessible to PNH patients are benefiting from a 10% price reduction – something that should align with PMPRB's objectives.

As taxpayers, we acknowledge that the PMPRB has done a good job over the past 30 years in fulfilling its mandate, helping to keep Canadian drug prices in line with those in Europe and much below those in the U.S. Yet today, other processes are in place at the pan-Canadian and provincial/territorial levels to ensure further cost savings are realized. CADTH and INESSS in Quebec both conduct rigorous clinical and cost-effectiveness reviews to inform public reimbursement decisions, and the pCPA conducts joint negotiations on brand name and generic drugs leading to further price reductions of approximately 30%. **While from the patient perspective these processes are far from ideal as they take a considerable amount of time, they at least result in most treatments eventually reaching the Canadian patients who need them.**

In undertaking this significant overhaul of the PMPRB, the fine balance between cost and effectiveness of drugs – especially those for rare diseases – has been disrupted. The concerns surrounding the proposed PMPRB Guidelines – their complexity, the discretion given to Board staff, the risk of an increased number of hearings, ongoing and increased litigation, and the focus on controlling manufacturers' revenues over protecting patients' lives – has had a chilling effect on the marketplace. The recent experience in PNH with new drugs coming to Canada is not unique: in 2019-2020, almost half (42%) of new drug launches in Canada were delayed for longer than six months following Health Canada approval, or were not launched here at all (compared to 22.5% in 2018-2019). **That means that the process of reforming the PMPRB is already having a quantifiable and detrimental, if not deadly, effect on patients' lives.**

As Canadians, we are fiercely proud and protective of our reputation as one of the most politically and economically stable countries in the world, with a universal healthcare system that is held in high regard. In the U.S. News & World Report *2020 Best Countries Report*, Canada was ranked #2 (and has been in the top three for the past five years) based on how global perceptions of characteristics, such as entrepreneurship and quality of life, define countries – impressions that have the potential to drive trade and directly affect national economies. **Why risk all that we have built and are respected for worldwide in a poor attempt to make one small part of a revered healthcare system appear more relevant and useful?**

Further, we question how those responsible for the delivery of healthcare across Canada feel about the impact that the proposed changes to PMPRB will most certainly have on their ability to save and extend the lives of people with rare diseases? PMPRB maintains that "orphan medicines are dominating the landscape" and are "pushing the limits of affordability," when in reality, drugs for rare diseases still only represent a very, very small portion of public drug costs

across Canada (approximately 2.5%). Until now, these medicines have been getting to patients who need them through public funding, but what happens when new life-saving and life-extending medicines stop coming to Canada because of the proposed changes to PMPRB? **What will our provincial and territorial governments say to their constituents who put their lives in their hands? Will they boast that thanks to PMPRB, they are now saving even more money on drug costs with fewer medicines to pay for?** We think not.

A decade ago, when I needed access to a life-saving medicine, our healthcare system – including PMPRB – allowed me to go on living and to become a grandfather to two beautiful children. With access to treatment, others in our community went on living, too, and were able to get married, have children of their own, build successful careers, and take advantage of all that Canada has to offer. **But make no mistake – if the uncertainty generated by the proposed PMPRB reforms had existed back when we needed access to clinical trials and treatment innovation, we would be long gone from this world.** Others, today, face this same fate, which is why it is our resolute position that anything which becomes yet another barrier for Canadian patients who need access to life-saving and life-transforming medications must be stopped.

Sincerely,

Barry Katsof
Founder & President
Canadian Association of PNH Patients