CANADIAN ASSOCIATION OF PNH PATIENTS

November 6, 2020

The Canadian Association of PNH Patients respectfully submits the following brief to the House of Commons Standing Committee on Health to inform the study it is undertaking on the Final Guidelines issued by the Patented Medicine Prices Review Board (PMPRB) on October 23, 2020.

Since the first Draft Guidelines were put forth by the PMPRB in November 2019, our community's concern has been that the proposed changes would severely limit access to new and innovative treatments, clinical trials and other global innovations in medicine for paroxysmal nocturnal hemoglobinuria (PNH) and other rare diseases. Nevertheless, our organization and many others representing thousands with rare diseases across Canada provided our input on those proposed changes, with the expectation that our voices would be heard. Yet, with the release of the final guidelines, it became evident that seemingly none of our input was reflected in the final content. As such, we remain extremely concerned that the health of Canadians living with PNH and other rare diseases continues to be put at risk by the uncertainty generated by the PMPRB reform process to date.

In the context of this regulatory reform, it is important that the Committee understands that without treatment – a life-threatening blood disorder – PNH has a death rate of more than 40% after five years. This is why 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.

Globally, as PNH patients we have reason to hope. 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 more than 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.

I know first-hand how important this research is to PNH patients, because a few years ago, the only approved drug for our disease stopped working for me. Soon after, I was fortunate to be enrolled in a clinical trial (at one of the two sites in Canada) for pegcetacoplan (APL-2), one of the most promising new molecules being studied today. Thankfully, I have responded well and, once again, my health has returned to "normal" for a healthy man of my age. Another member of our PNH community, Olivia Oliverio, who just turned 24 and lives in Alberta, also failed on the only treatment accessible in Canada for PNH and is now enrolled in the same clinical trial. Here's a small, but very relevant, part of her story:

> *"In 2017, I was diagnosed with PNH, a very rare and debilitating disease of the bone marrow that affects the blood and major organs. I had to adjust my lifestyle to deal with the new adversities I was forced to face by my illness. I learned the hard way that the only treatment that was approved for PNH and accessible in Canada had stopped being effective for me when I was participating in a dance competition and my hemoglobin dropped to a critically low level. I still don't know how I competed on stage – I remember feeling like the life had been sucked out of me. And, I felt so alone. I had an extremely rare disease and the only treatment for it was no longer working – something that even my doctor had never experienced with any of their patients.*
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> *After about a year-and-a-half of living from blood transfusion to blood transfusion, a new clinical trial came to Canada that was made for people exactly like me. I was quickly registered for the trial and started receiving the medication that turned my life around. The medication has been so effective that I now lead a relatively normal life.*
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> *The proposed changes to the Regulations governing the PMPRB would inhibit my access to the only medication that allows me to continue living. In addition, they would prevent new clinical trial sites from opening in Canada which could help others like me. If I am unable to receive my medication, the ramifications are detrimental and even deadly.*
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> *Without access to my current treatment, I would need constant blood transfusions to keep me at a "livable" level. My productivity in all aspects in my life would decline, as I would be so fatigued that the simplest of tasks would seem impossible. I would not be able to think clearly and focus, which would have a negative impact on my job. My desire and ability to go out and be social would decrease significantly, impacting my*

> *relationships and my mental well-being. Without access to my medication, I would not be able to do one of the most important things in my life, dance.*
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> *I have lived life without proper treatment, and I do not want to regress. I would have to face unnecessary barriers that I know I do not have to face because there is a treatment out there that works for me. After living with life-threatening rare diseases for nearly a decade, I have received countless medications through public funding that if I were not able to receive, I would not be here today. In short, access to my current medication for PNH is vital to my life.* ”

In another 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 at a time when it is most needed – during a pandemic. This treatment (ravulizumab), 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 option, 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. Without access to this new treatment, not only are Canadian patients missing out on a significant improvement to their quality of life, but public payers are also missing out on a 10% cost savings (compared to the existing treatment) and further savings from a reduction in clinic visits. This could be a win-win situation for all.

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.**

A real-time example of how uncertainty is impacting our market is unfolding right now with the molecule I mentioned earlier – pegcetacoplan – poised to become the second novel treatment approved globally for PNH. The manufacturer recently announced submissions to the FDA and the EMA, and that pegcetacoplan has been granted orphan drug designation for the treatment of PNH patients in Australia. Yes, Australia – a country with a smaller population and GDP than Canada, but with favourable regulatory, pricing and reimbursement policies that support swift access to rare disease drugs. What the company has not yet announced is when, or if, the drug will be launched here in Canada. As you can imagine, patients like Olivia and I, who are fortunate to be doing well on this treatment in a clinical trial setting, are concerned that others in Canada may not have access to this lifesaving drug.

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%).

As a PNH patient, I am elated by the unprecedented number of potential treatments for PNH currently in development worldwide. And, in countries where drug pricing regulations and legislation exist to support reimbursement, I am thrilled to see that lives are being improved, extended and saved. But as a Canadian, I have genuine concerns that the proposed amendments to the PMPRB, if rushed through by the end of the year, could significantly delay medications coming to our country, result in decreased research and development by the pharmaceutical industry and, worst of all, cause critical treatments to not even be launched in Canada. These concerns are shared by many in our community, like 24-year-old Audrey Lagacé, a Registered Nurse from Québec:

> *"I was diagnosed with PNH a month after my 13th birthday. I was told that without treatment, I would probably only live another 10 to 15 years and that I was at risk of dying at any moment due to blood clots. The doctors did know there was a medication out there – eculizumab– that could help deal with my symptoms and improve my life expectancy, but unfortunately at that time it was not publicly funded by provincial drug plans.*
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> *My health would deteriorate in the years that followed with more and more hospitalizations. My symptoms were becoming more severe and my body became unable to function. Thankfully, after a few years of fighting government and insurance companies, I finally got access to eculizumab. I feel so lucky to have been able to receive this treatment because, without it, I would never have been able to lead a normal life and pursue my studies to become a Registered Nurse. It's been 10 years since I was first diagnosed with PNH, and I am thriving! I work as a nurse at Charles-Lemoyne Hospital in the Hematology-Oncology Department. I am able to do this, and be the woman I am today, because I got the treatment I needed. I know that without it, chances are, I would not have made it this far.*
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> *That is why I'm frightened by the new PMPRB guidelines. I already had the hardest time getting access to the only medication currently available in Canada to treat PNH. What if Soliris stops working for me, as it has for other PNH patients, and I need a new treatment option? I might never have access to new treatments or clinical trials for possible cures. That would mean the Government of Canada would be taking away my life; not only my life, but the lives of thousands of people suffering from rare illnesses that are difficult to treat and have limited research.*
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> *I ask you to stop and think about how PMPRB reform might affect the lives of those of us here in Canada, living with rare conditions that are just as difficult and important as diabetes, cancer, heart disease, etc. Keep in mind that **none** of us asked for this. All we ask for is the possibility to live our lives and to be a part of society."*

A decade ago, when I needed access to a lifesaving 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 – like Audrey – 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, many of us would be long gone from this world.** Today, others 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 lifesaving and life-transforming medications must be stopped.

CANADIAN ASSOCIATION OF PNH PATIENTS

Sincerely,

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