

June 23, 2017

Patented Medicines Regulations Consultations
70 Colombine Driveway, Tunney's Pasture
Floor 10, Brooke Claxton Building
Ottawa, Ontario K1A 0K9

To whom it may concern:

The Canadian Association of PNH Patients respectfully submits to the following response to the Health Canada consultation currently underway regarding proposed amendments to the Regulations governing the Patented Medicine Prices Review Board (PMPRB). Our stakeholder group supports the needs of Canadians living with a life-threatening rare disease called paroxysmal nocturnal hemoglobinuria (PNH). Our response to the five proposed amendments outlined in the consultation document is reflective of the fact that there is currently only one treatment available and accessible to Canadians with PNH, and while it is costly, this medication saves patients from certain death. As such, we view any new or existing barriers to accessing Health Canada-approved treatments for PNH as unacceptable to our patient community, as outlined below in responding to the consultation questions.

- The factors that are important to patients of all kinds are the availability of and access to medications that are effective, safe, and convenient to take (i.e. an oral drug is preferable to an intravenous infusion) which supports patient adherence and improved health outcomes. The same factors are important to patients with rare diseases, but in addition, they are concerned that they are not denied access to treatment because the health system balks at paying the high prices for drugs that are the only options they have to extend and save lives, and transform the quality of their lives.
- When the number of potential patients in Canada is small, as is the case for drugs for extremely rare diseases, the small market justifies a high price. If a new drug for a rare disease has a high level of efficacy and is for a disease for which there is no current therapy or the present treatment is ineffective, a high price is justified. Patients should not be denied life-transforming treatment because the PMPRB has “sticker shock” over the cost of saving a life.
- The health technology assessment (HTA) of the “value” of a new drug is already performed by the Canadian Agency for Drugs and Technologies in Health (CADTH). However, CADTH’s assessments are not effective in the evaluation of drugs for rare diseases,¹ leading to delays or denial of access to these medications in the public drug plan system. An additional requirement for manufacturers to submit another HTA to the PMPRB is duplicative work for companies and government, which will cost taxpayers more money and further restrict patient access to essential treatments. As such, manufacturers should not be required to submit additional pharmacoeconomic evaluations to the PMPRB. Instead, the current HTA process through CADTH should be improved upon to better safeguard the health all Canadians, including those living with a rare disorder.

¹ Rawson NSB. Are the cost-effectiveness rules used by public drug plans denying coverage to Canadians with rare disorders? *Canadian Health Policy*. Toronto: Canadian Health Policy Institute, 2015. <http://www.canadianhealthpolicy.com/products/are-cost-effectiveness-rules-in-public-drug-plans-denying-coverage-to-canadians-with-rare-disorders-html>; Rawson NSB. Health technology assessment of new drugs for rare disorders in Canada: impact of disease prevalence and cost. *Orphanet J Rare Dis* 2017; 12: 59.

- Any requirement for HTAs from other countries or the disclosure of prices in other countries will impact the introduction of drugs into other jurisdictions and will either result in companies launching drugs later in Canada, or not launching them at all. This outcome would delay or deny patient access and, consequently, put their lives at risk.
- Any perceived association between drug prices and GDP is irrelevant to patients. If the government attempts to link increases in drug expenditure to increases in GDP, this would place patient access to new drugs in jeopardy. Patient health should not depend on the growth of the Canadian economy.
- The PMPRB has done a good job over the past 30 years. It must not be revised so that it becomes yet another barrier for Canadian patients who need access to life-transforming and life-saving medications that are effective, safe, support adherence and, for patients with rare diseases, frequently the only treatment option available.

We thank Health Canada for the opportunity to contribute to this consultation process and look forward to seeing our views reflected in the regulatory proposal and the Regulatory Impact Analysis Statement, as indicated in your consultation document.

Sincerely,

A handwritten signature in black ink, appearing to read "Barry E. Katsof". The signature is fluid and cursive, with a horizontal line extending from the end.

Barry E. Katsof
Founder & President