1. *What does the word "excessive" mean to you when you think about drug pricing in Canada today?*

When used by an individual or government body involved in drug pricing and reimbursement processes in Canada, the word "excessive" usually means an unwillingness to pay based on an assumption of greed, which unfortunately for patients who depend on public drug funding, gravely impacts their access to life-transforming or life-saving treatments.

According to its mission statement, the Patented Medicine Prices Review Board (PMPRB) exists, in part, to provide stakeholders (i.e. payers) with information to help them make "timely and knowledgeable pricing, purchasing and reimbursement decisions" for medicines that have been approved by Health Canada as safe and effective. The current reality, and we hope the real reason the PMPRB is revisiting its guidelines, is that "timely" and "knowledgeable" are in conflict in this mission statement – a conflict that often results in delayed, severely restricted or outright negative drug funding decisions.

The vision of the PMPRB includes ensuring Canadians have access to patented medicines at affordable prices[1]. To realize this vision fairly and equitably to the benefit of all Canadians, it is important that the PMPRB takes into account the uniqueness of those drugs that are approved to treat rare diseases and disorders, and fully understands, accepts and reflects through their policies the important differences between these drugs and others that treat common diseases.

In Canada today, one in 12 Canadians, two-thirds of which are children, are affected by a rare disease[2]. The minute size of rare disease communities combined with the higher per-patient cost of drugs developed to treat them makes reimbursement decisions more complex than for drugs that treat millions of patients with more common diseases. It also necessitates a pricing formula that isn't corrupted by "sticker shock," but takes into account the overall cost of funding these drugs – a mere drop in the bucket of any drug plan's budget.

As a businessman, I understand that drug manufacturers need to operate within a pricing paradigm that ensures an adequate return on investment, and allows for reinvestment in the research and development needed to bring more beneficial drugs to patients. Without an appropriate pricing paradigm for rare disease drugs in Canada, advances in innovation will never make it here and lives will most certainly be lost. In fact, that is already a sad reality in some cases.

As founder and leader of a rare disease community and a patient myself, **one thing matters to me above all else – that nothing stands in the way of getting approved, life-saving drugs to those who need them**. It should be the only thing that matters to the PMPRB and any government body whose reimbursement recommendations and decisions it impacts. We need to get past internal biases that lead to blinding outrage around drug pricing and refocus on patient need. In undertaking this review of its guidelines, it is up to the PMPRB to figure out how to make it all work – but not at the expense of human lives.

[1] http://www.pmprb-cepmb.gc.ca/en/news-and-events/consultations/current-major-consultations/rethinking-the-guidelines/discussion-paper
[2] https://www.raredisorders.ca/our-work/

In your questionnaire, you ask if it matters to me if a very costly drug only treats a small group of patients, such that it accounts for a very small proportion of overall spending on drugs in Canada. As stated, yes, without question the number of patients a drug can benefit absolutely should be considered when reviewing price, because it determines the overall cost to payers.

On its website, the PMPRB lists the factors that affect the cost of prescription medicines in Canada (see list below). If one of the factors it takes into account is the number of drugs used per patient, doesn't it then make sense to factor in the number of patients who could use a given drug, and whether that drug is the only one available to treat their disease?

Factors that influence the total cost of drugs:[3]

- prices of patented drugs
- prices of non-patented drugs
- prices of generic drugs
- retail and wholesale mark-ups
- pharmacists' professional fees
- changes in the composition of total population, e.g. proportion of older persons
- changes in prescribing habits of physicians
- **changes in the utilization of drugs, i.e. number of drugs used per patient**
- **trends towards using newer drug therapy instead of other treatments**

From the perspective of a patient fighting a life-threatening, unpreventable rare disease, I do not see how it can or should be the role of the PMPRB to, in essence, play "God" by making or affecting life-or-death decisions. That is exactly what it is doing by overlooking key factors that are unique to drugs that treat rare diseases as noted above.

2. *Given that it is standard industry practice worldwide to insist that public prices not reflect discounts and rebates, should the PMPRB generally place less weight on international public list prices when determining the non-excessive price ceiling for a drug?*

When setting a maximum price for drugs, it is critical to compare "apples to apples." If the standard industry practice worldwide to determine the non-excessive price ceiling for a drug took into account the above-mentioned attributes unique to rare disease drugs and thus enabled access, rather than restricted it, patients would benefit. The problem in Canada is that currently only 60 per cent of treatments for rare disorders make it to Canada, and most of those are approved up to six years later than in the US and Europe.[4] Of those drugs, just over half are publicly funded. Clearly, standard industry practice is not working for rare disease drugs as it is currently applied.

[3] http://www.pmprb-cepmb.gc.ca/about-us/frequently-asked-questions
[4] https://www.raredisorders.ca/our-work/

3. In your view, given today's pharmaceutical operating environment, is there a particular s. 85 factor that the Guidelines should prioritize or weigh more heavily in examining whether a drug is potentially excessively priced?

The number one priority in guiding drug funding decisions must be efficacy. If an approved treatment has been shown to improve, extend or save lives, then the PMPRB has a responsibility, as per its vision and mission stated above, to ensure that treatment is not withheld from patients due to price.

4. Should the PMPRB set its excessive price ceilings at the low, medium or high end of the PMPRB7 countries (i.e., the US, the UK, Sweden, Switzerland, Germany, France and Italy)?

As stated above, there cannot be a one-size-fits-all process. The formula must consider the patient, the disease and the need for treatment. If a drug is scientifically proven to save lives, then it is not the role of the PMPRB to prevent that treatment from being accessible to patients.

5. Does the amount of research and development that the pharmaceutical industry conducts in Canada relative to these other countries impact your answer to the above question and if so, why?

No. Regardless of where R&D money was invested, patients worldwide should be provided the benefit of the resulting innovations in therapy. The lack of R&D investment in Canada should not be used by the PMPRB or any government body to punish patients living in this country.

6. What alternatives to the current approach to categorizing new patented medicines (based on degree of therapeutic benefit) could be used to apply the statutory factors from the outset and address questions of high relative prices, market dynamics and affordability?

As stated above, any formula or approach applied must consider the patient, the disease and the need for treatment. If a drug is scientifically proven to save lives, then it is not the role of the PMPRB to prevent that treatment from being accessible to patients.

7. *Should the PMPRB consider different levels of regulatory oversight for patented drugs based on indicators of risk of potential for excessive pricing?*

The focus cannot simply be on price – efficacy should be the priority. Canada is lagging behind when it comes to rare disease policies and currently, the total amount spent on drugs for these patients equates to only one per cent of public drug budgets. Processes like the pan-Canadian Pharmaceutical Alliance (pCPA) are in place to facilitate the negotiation of acceptable drug prices. **As stated above, any government bodies involved in drug approval, pricing and reimbursement must focus on saving lives over saving money, or find a way to do both.**

8. *Should the price ceiling of a patented drug be revised with the passage of time and, if so, how often, in what circumstances and how much?*

As stated above, the pharmaceutical industry must be given the tools to continue to conduct research and develop important treatments that advance innovation, and no government body should impede that progress, or patient access to its potentially life-saving benefits.

9. *Should price discrimination between provinces/territories and payer types be considered a form of excessive pricing and, if so, in what circumstances?*

N/A

10. *Are there other aspects of the Guidelines not mentioned in this paper that warrant reform in light of changes in the PMPRB's operating environment?*

In order for the PMPRB to realize its vision of ensuring Canadians have access to patented medicines at affordable prices, and carry out its mission to provide information that informs timely and knowledgeable pricing, purchasing and reimbursement decisions, it must make the health of patients a priority over containing costs.

Again, as a rare disease patient, I respect the need for the affordability of drugs, but I believe that a fair and equitable formula, which accounts for the rare disease community can and will accomplish this.

11. *Should the changes that are made to the Guidelines as a result of this consultation process apply to all patented drugs or just ones that are introduced subsequent to the changes?*

N/A

12. *Should one or more of the issues identified in this paper also or alternatively be addressed through change at the level of regulation or legislation?*

As a result of the pharmaceutical industry's reinvestment in research, enabled by public and private drug funding, the science is getting better and the number of targeted, specialty medicines is consistently increasing, as noted in your discussion paper. Many of those treatments are now being discovered for rare diseases, and where drug pricing regulations and legislation exist to support reimbursement, lives are being improved, extended and saved. **Anything that can be done in Canada to improve patient access to these innovative treatments, must be considered and adopted expeditiously.**