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

February 12, 2020

Patented Medicines Pricing Review Board
Brooke Claxton Building
70 Colombine Driveway, Tunney's Pasture
Mail Stop 0910, Floor 10,
Ottawa, Ontario K1A 0K9
PMPRB.Consultations.CEPMB@pmprb-cepmb.gc.ca

Re: PMPRB Draft Guidelines Consultation

To the Patented Medicines Pricing Review Board:

The Network of Rare Blood Disorder Organizations (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 acknowledge the unique role the PMPRB plays protecting Canadians from excessive drug prices, and welcome this opportunity to provide feedback on behalf of patients on the proposed amendments to the patented medicines guidelines to enforce the updated regulations. We were also grateful to participate in the consultation session in Ottawa on December 10.

Timely and equitable access to drugs and biologics is an important issue for our member organizations and the patients they represent. These patients rely on prescription therapies to manage their rare blood disorders and improve their quality of life, and as such they also rely on an effective and sustainable pricing and reimbursement system.

The NRBDO board and membership are sensitive to the potential barriers these new regulations could create, delaying access or discouraging the introduction of innovative therapies to the Canadian market altogether. While we are hearing warnings from industry and other concerned civil organizations, we have not yet come across a third party endorsement for these draft guidelines with regards to the treatment of orphan drugs and we heard nothing at the December 10th consultation in Ottawa that was reassuring either.

As an example, a new therapy for the treatment of PNH is currently listed as “paused” on CADTH’s website. We are told that this is at the request of the manufacturer, and is a direct result of the new PMPRB Pricing Regulations which may cause the manufacturer to delay bringing this therapy to Canada. We have no way of knowing if this is a ploy, or if this will be the

new normal for our member patient groups, so we are watching these guideline developments closely.

Pharmacoeconomic evaluations based on QALY comparisons are of concern to those with rare diseases, even with the proposed rare disorder market size adjustment in Section V. As you have acknowledged, the QALY, while a useful tool in some arenas, does not account well for small patient populations, and will not prove to be an appropriate measuring tool for price setting in these instances. Indeed, even with a 50% adjustment as proposed, it is possible that very few of the life-saving therapies taken by the patients we represent would have even come to market with this proposed formula.

We are not pharmacoeconomicists, and we do not have the magic formula. We do however ask that you give every consideration to the potential impact these guidelines will have on Canadians living with rare disorders, and be open to adjusting the formula should unintended access issues arise as a result.

Many thanks again for the opportunity to participate in the guidelines consultation.

Sincerely,

Erin Harder
Acting Chair, NRBDO