Canadian Organization for Rare Disorders

December 5, 2022

Patented Medicine Prices Review Board (PMPRB)
333 Laurier Avenue West, Suite 1400
Ottawa, Ontario K1P 1C1

RE: 2022 PMPRB Guidelines Consultation

Dear Members of the PMPRB,

The Canadian Organization for Rare Disorders, in collaboration with the federal and provincial governments and other stakeholders, is looking forward to the imminent implementation of Canada's Rare Disease Drug Program, announced in 2019 by the federal government with a commitment of $1 billion over two years starting in 2022-2023. The vision is sustainable access to rare disease treatments, which means delivery of optimal health outcomes to persons with rare diseases as well as optimal returns on investment to society. Core to achieving this vision is investment in rare disease infrastructure, namely, timely and accurate diagnosis, access to centers of excellence with specialists networked to localized health professionals close to the patients and families. Through these networks, rare disease patients will be prescribed "individual" best therapies, including innovative medicines, with assessment of real-life impact (good and bad) and treatment adapted appropriately.

The effectiveness of the Patented Medicines Prices Review Board (PMPRB) guidelines can only be assessed against the delivery of optimal outcomes to patients, which, in turn, will assure that health and other support resources are used appropriately and most cost-effectively. To that end, investment in all of the elements of the health ecosystem must be considered jointly, not as siloed budget items or *"cost centres."* Indeed, the notion of a "fixed" drug budget within a health system or a private drug plan is an anachronism that does not reflect the requirements of "modern" medicines for a coordinated program of targeted diagnosis, specialist prescribing and long-term drug management. The operation of a single agency adjudicating drug prices without consideration of the value to patients, the health system, and the society is not only counterproductive but dangerous.

Since the PMPRB's "so-called" reform process began in 2017, CORD has actively raised the concern that the PMPRB's singular focus on driving medicine prices as low as possible has had and will increasingly impact the entry of new medicines into Canada, as demonstrated by the number of clinical trials and new drug submissions. This has been especially damaging to individuals living with rare diseases with progressive and life-threatening conditions for which there are few or no effective therapies. But we have warned you about this repeatedly, ad nauseum, with seemingly no recognition or impact.

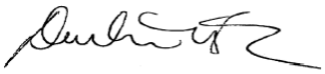
Over the past couple of decades, science and technological innovation have generated life-saving and life-altering medicines not only for rare diseases but many common conditions, often based on an understanding of the underlying genetic defect and how to correct it. The results can be long-lasting and, indeed in some cases, a complete elimination of the disease, that is, a cure.

CORD has responded to every PMPRB consultation on revised guidelines, including revisions of revisions of proposed guidelines. We have participated actively in a multi-stakeholder Advisory Board and submitted detailed responses to each and every consultation.

With all due respect, we request that the PMPRB cease and desist with these endless revisions that do not address the core need: sustainable access to the best therapies, investment in Canadian research and development of new therapies, and prices that reflect the return on investment for individuals, families, health systems, and society.

We look forward to your response.

Sincerely,



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