Standing Committee on Health:
Written brief for the study on the Patented Medicines Prices Review Board's Final Guidelines

By: ALSAC – ALS / SLA Action Canada

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Standing Committee on Health
Sixth Floor, 131 Queen Street
House of Commons
Ottawa ON K1A 0A6
Canada

Dear Members of the Standing Committee on Health,

On behalf of ALS/SLA Action Canada, we are pleased to provide a written brief for the study on the Patented Medicines Prices Review Board's Final Guidelines issued on 23 October 2020. ALS/SLA Action Canada is a new patient-led initiative advocating for urgent access to promising therapies for Canadians living with Amyotrophic Lateral Sclerosis (ALS).

INTRODUCTION

We write to you as Canadians with ALS: We are the nation's doctors, firefighters, lawyers, teachers, business owners military members and tradespeople. The day we were diagnosed with ALS was the day we were sent home to die.

Eighty per cent of people with ALS die within two to five years of being diagnosed. The 20% who live longer usually rely on a ventilator through a tracheotomy, communicating only with their eyes, described by the ALS community as living in a 'glass coffin'. ALS is unique among other diseases, both rare and not, in its rapid progression and 100% fatality.

OUR PERSPECTIVE

There is new hope for all people with ALS given that over 160 pharmaceutical companies worldwide are working on ALS therapies and treatments, and several promising clinical trials are in their final stages, including one that may stop the progression of ALS.

Sadly, the vast majority of ALS-related clinical research and trials are occurring outside of Canada. This must change and change quickly. We need to have access to these promising therapies, and those to be developed in the future, within Canada. For this to occur, it is imperative that pharmaceutical companies see Canada as a viable market to run clinical trials and pursue regulatory approval. The reality today, to which the proposed guidelines of the PMPRB if enacted will exacerbate access for patients like us. This means that a very small and extremely vulnerable group of Canadians with a rare and 100% fatal disease, we lost out on possible treatment within our own borders.

It is our understanding that the PMPRB, and the supporting legislation, was originally established to ensure that the prices of patented medicines sold in Canada are not excessive. We appreciate that the Canadian government is committed to a national pharmacare program, and that the PMPRB efforts are expected to yield greater pricing efficiencies nationwide. However, efforts intended to lower prices must be made in a balanced way that continues to encourage innovation, takes into account our very vulnerable ALS population, and does not result in the delay of launches and introduction of new medicines in the Canadian market.

There needs to be collaborative outreach to relevant stakeholders, including patient representatives, to ensure equal consideration is given to a drugs ability to provide the best

possible health, physical function, and lifespan despite being afflicted with ALS. This holistic approach should also take into account the opportunity costs of not attracting therapies to Canada – the significant, ongoing health care costs arising from the effects of our devastating disease.

In a recent Letter to the Editor in *The Hill Times*, the President of Innovative Medicines Canada (IMC), Pamela Fralick stated:

> "Recent data from a range of sources confirms what Canada's innovative medicines industry has been saying for more than three years now: Health Canada's recent changes to the PMPRB will have a negative impact on patients' access to medicines, on investment in our life-sciences sector, and on the launch of new medicines and vaccines in Canada."

Ms. Fralick further articulated that "since the amended PMPRB regulations were published last August, IMC members have reported several drug-launch delays or suspensions, including for rare disease and oncology medicines.

The current Canadian drug approval process is longer than the expected lifespan of an ALS patient. Health Canada maintains a six-month review process similar to the FDA and EMA. However, Canada is not always pharmaceutical companies' first choice for launching therapies, and on average, there is a nine-month delay between a drugs' FDA approval and a subsequent application to Health Canada.

Canadian patients often watch larger markets like the US and the EU access treatment fifteen months before them. Following this, the provincial drug coverage process degenerates into a province by province approval process, decentralized and uncoordinated, failing to appreciate the life-expectancy of an ALS patient.

The fastest provincial drug coverage approval for an ALS medication to date, was 574 days in Quebec. **Between federal and provincial drug approvals, some patients wait 34 months to access new therapies.** In other words, if a scientifically validated cure is made available in the US tomorrow, and a Canadian is diagnosed with ALS the following week, that Canadian patient may not survive to access the therapy. They will most certainly have to pay for it and if they cannot afford the therapy, they, in all probability, will die before provincial coverage is made available. Canadians are fighting for the right to live.

OUR POSITION

We respect PMPRB's consumer price protection mandate and legislative intent to reduce the cost of patented medicines, with one caveat: we are a small group of terminally ill Canadians with rapid progression. For us, ensuring the implementation of the proposed PMPRB guidelines

does not impede urgent access to promising therapies for ALS, is literally a matter of life and death. We therefore support the following recommendations:

1. That the Federal Government require PMPRB to engage an independent third party to conduct a formal assessment of the real-time and potential impacts of the reforms on access to therapies and research investment in Canada (including clinical trials), with specific consideration to therapies for rare diseases, before the PMPRB guidelines are implemented.
2. That the PMPRB undertake a phased approach to enacting proposed reforms as contemplated under the formal assessment as set out in 1. above. This ensures the impact of each change on research investment and access to therapies for both rare and non-rare disease therapies can be fully assessed. Only then should additional reforms be considered. For people living with ALS, the impact of these changes has life and death consequences. We therefore further ask that the PMPRB compassionately provides any and all potential drugs and treatments for ALS with an exception status to PMPRB regulations.
3. That the PMPRB implements a distinct pathway for medicines for rare disease, recognizing, a) the devastating implications of our disease requires rapid approvals, b) the need for specialized expertise to provide input to the decision-making process, and c) the small patient population associated with such drugs imposes a relatively nominal impact on the overall health care spending in Canada.
4. That the Federal Government require that PMPRB decision-making and processes include patient representatives.

We sincerely thank you for your consideration of our recommendations.

Yours truly,

Deane Gorsline
Chair, ALS Action Canada