August 4, 2020

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

Dear Members of the PMPRB,

This feedback on the revised PMPRB Guidelines of June 19, 2020 is being provided on behalf of ALS/SLA Action Canada, a new patient-led initiative advocating for urgent access to promising therapies for Canadians living with ALS.

INTRODUCTION

We write to you as Canadians with Amyotrophic Lateral Sclerosis (ALS). We are the nation's doctors, firefighters, lawyers, teachers, business owners, executives, military members, and tradespeople. The day we were diagnosed with ALS 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 drug companies worldwide are working on ALS therapies and treatment, and that 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, is that Canada has barriers to market access that other G7 countries do not. This means that as a very small and extremely vulnerable group of Canadians with a rare and 100% fatal disease, we lose 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.

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 don't 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

Cc: Francis Drouin, MP, Chair, ALS Caucus
Ms. Tammy Moore, CEO, ALS Canada