August 15, 2021

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

Dear Members of the PMPRB,

This letter provides feedback as our response to PMPRB consultation underway on the new Guidelines slated to come into effect January 1, 2022.

INTRODUCTION

As organizational context, ALS/SLA Action Canada, is a patient-led initiative advocating for urgent access to promising therapies for Canadians living with ALS: Amyotrophic lateral sclerosis. We collaborate with other ALS organizations provincially, nationally and globally. Unlike other organizations our main focus is to enable rapid access to potential therapies for Canadians with ALS, a disease that currently has no effective treatments in Canada.

Disease and Canadian population context: ALS is a currently 100% terminal disease. Approximately 3,000 Canadians have ALS at any one time. Eighty percent of Canadians 20 years old and up pass away within 2-5 years of diagnosis. 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'. There is a one in three hundred chance of receiving an ALS diagnosis without any prior family history.

Treatment context for Canadians with ALS: There are no effective treatments available for ALS to slow or stop this terminal and fast-moving disease. In fact, since writing to you last year, one of our members and contributor to our August 8, 2020 letter, Mr. Kerry Winkler, has passed away. We provide our feedback to you, thinking of our friend Kerry and his efforts to try and help gain rapid access to potential ALS therapies for affected Canadians.

We write to provide this feedback with the understanding that while the current drug treatment options are few to none, the horizon looks promising with over 160 clinical trials underway worldwide.

OUR POSITION

Canadians with ALS are in a tight spot:

- As a lethal disease, 3000 Canadians have a diagnosis of ALS at any one point in time: 1000 Canadians are newly diagnosed each year and 1000 die each year.
- The disease is currently terminal and most often moves rapidly: in 2021, no one survives ALS
- The stakes for Canadians with ALS are high: each person has an individual right to treatment/drug access given the severity of the disease.
- While this is an unprecedented time of hope and clinical trial activity, drug treatments are just finishing clinical trials: there are no treatments to stop or slow the disease
- Canada's drug approval process is longer than the life of a person diagnosed with ALS
- Pharmaceutical and biotech companies report a more welcoming, less burdensome drug approval process in other jurisdictions such as Europe and the UK
- Canadians with ALS want treatment options in Canada: We have laws that allow people with lethal diseases to die with dignity. Surely, they should also have the right to rapidly access medicines that could help extend their lives with good quality… or turn their lethal diseases into chronic, manageable conditions, or better.

We are asking PMPRB to:

1. **RE-VISIT IT'S GUIDELINES** to make them less complex. ALSAC finds the guidelines too complicated to understand with respect to assessing new drugs. Promising new drugs are the future and hope for Canadians with ALS. Its our view that overly complex and unclear guidelines for new drugs will deter potential new pharmaceutical companies from making application for drug approval to Canada. Further, if pharmaceutical companies do not foresee the potential for approval in Canada, they are not and will not bring their clinical trials to Canadians with ALS.

2. **REVISIT and EXERCISE YOUR MANDATE WITHIN THE LAW/JURISPRUDENCE:** Ensure decisions regarding potential ALS drugs/treatments are limited to PMPRB's Section 85 Patent Act Excessive Pricing mandate: Consistent with your lawful mandate to review drugs for Excessive Pricing, and fully consider the Costs of making and marketing potential drugs/treatments for ALS. We understand that PMPRB Guidelines are non-binding and cannot affect Section 85 of the Patent Act.

    a. *Consider all drug development life-cycle costs when assessing excessive pricing:* All pharmaceutical companies bring ALS drugs or treatments have borne significant and long-term investments in the drug trialling, research and development processes. We want to ensure PMPRB considers all of those costs prior to any 'excessive cost' decision. Developing ALS drugs/treatments is expensive.

b. *PMPRB should revisit its list of comparator countries to ensure Canada's pricing approach is transparent and reasonable.* There needs to be room for pharmaceutical companies to negotiate a fair price that allows Canadians with ALS to rapidly access potential new therapies. Canada is not a median OECD country. It is in the higher range of per capita average wages for OECD countries: https://data.oecd.org/earnwage/average-wages.htm. Canada is ranked eighth out of 36 countries. Companies will not be happy to sell Canada their new drugs for much less than they get in about half of the other 36 OECD countries. New PMPRB regulations would have a significant negative impact on product launches and supply, compassionate access programs, research, employment and manufacturing. This threatens not only access to innovative, effective lethal disease treatments but also our economy. Drug prices should fit the market.

3. **UNDERSTAND THAT TIME IS OF THE ESSENCE FOR ALS:** We ask the PMPRB prioritize any drug or treatment for ALS for fast-track priority decision making. Take a risk-based approach to processing potential treatments and drugs for ALS given its fast-moving and current status as terminal disease. Time is of the essence: risk has a different meaning if your facing death.

   A terminal disease requires broadened assessment parameters than other diseases.

   a) Pharmaco-economic considerations should not be the main drug approval consideration: we are talking about individual Canadians, their families and communities
   b) Parameters should include patient and caregiver quality of life improvement, resulting from the use of a promising new drug
   c) The cost of palliative and long-term care of a Canadian with ALS to the health care system will exceed the cost of a promising new drug which would improve the function and quality of life for the person living with ALS, their families and communities.

4. **SEEK ALS EXPERTISE FOR SCIENTIFIC/MEDICAL REVIEW:** Understand the narrow and limited knowledge of ALS as a rare and terminal disease in Canada. This means that any 'expert' review of the drug or potential treatment under consideration by PMPRB might be difficult to find without encountering a conflict of interest. As group of Canadians with ALS we are offering to identify non-Canadian clinicians and scientists recognized by the global ALS community that you might want to consult in the review of any potential ALS drug/treatment.

We sincerely thank you for your consideration of our recommendations.

Yours truly,

Jm per

Deane Gorsline
Chair, ALS Action Canada
Cc: Francis Drouin, MP, Chair, ALS Caucus