Dr. Mitchell Levine
Chairperson
Patented Medicines Prices Review Board
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Ottawa, Ontario K1P 1C1
email: PMPRB.Consultations.CEPMB@pmprb-cepmb.gc.ca

RE: ALS Canada Written Submission in Response to the PMPRB Draft Guidelines Consultation

Dear Dr. Levine:

In representing the thousands of Canadians living with ALS, this letter is in support of the recommendations put forward by Health Charities Coalition of Canada (HCCC) in response to the Patented Medicine Prices Review Board (PMPRB) public consultations on its draft Guidelines to implement the new *Patented Medicines Regulations.*

The ALS Society of Canada (ALS Canada) supports the improvement of equitable, timely and affordable access to therapies for the approximately 3,000 Canadians living with ALS. While we recognize the government's goal of lowering pharmaceutical drug prices, we urge you to understand that these draft Guidelines will result in fewer innovative therapies in Canada if implemented in their current form.

With no cure and few effective treatments, 80% of people living with ALS die within two to five years. This is the devastating reality faced by our community. Receiving an ALS diagnosis is something no person ever wants to hear because it means death within a matter of months or years. Even more devastating, however, is knowing that there is an existing therapy that can slow, stop, or even reverse the symptoms of ALS, but you cannot access it because of where you live.

Our community has already experienced this when they had no choice but to witness people in the US gain access to the second ALS therapy ever for over a year before it came to Canada to start the approval process. And now, the reimbursement decisions have further delayed access more than a year post-approval. With more phase 3 clinical trials than ever before leading to new ALS therapies, we are concerned that the draft Guidelines will exacerbate this problem.

We also want to reiterate HCCC's disappointment about the fact that patient voices were marginalized during the lengthy consultation process and that initial concerns raised in 2017 remain unaddressed. Previous efforts to communicate with the Government of Canada over the past two years about the harm that these changes will have on people and families living with ALS have seemingly gone unheard.

It is with grave urgency that we call on you to listen to the perspective unique to the people and families affected by ALS and implement the recommendations outlined in this letter and the written submission from HCCC:

HCCC Recommendation #1: *That the PMPRB undertake a stepwise approach to its proposed changes by initially enacting only the changes to the comparator countries. Once the impact of this change is fully understood and if the objective of lowering Canadian prices sufficiently has not been met, then other new elements could be considered.*

- **Impact on Affordability:** Affordable access to therapies is vital for people living with ALS, but for a community that continues to face a devastating terminal disease, this cannot be achieved at the expense of timely access. Efforts intended to lower prices must be done in a balanced way that continues to encourage innovation and does not result in the delay of launches and uptake of new medicines in the Canadian market. We must be able to fully realize the impact of the initial change to the basket of comparator countries, particularly any impact on the availability of new therapies, before implementing further measures. Taking an incremental approach is crucial to making smart pharmaceutical policy changes that have the best possible impact on patients.

HCCC Recommendation #2: *That a multi-stakeholder dialogue be established to evaluate the impact of the changes on availability of medicines and specifically to inform any decision on whether and how to implement the use of the new economic criteria.*

- **Impact on Access:** The need for timely access to therapies for people living with ALS is immense. This is a community that measures time not by weeks and months, but by loss of movement and loss of life. Significant decreases in price will lead to delays in manufactures launching their product in Canada, meaning new life-saving drugs could be available in other countries before they are available in Canada. This puts people living with ALS in a position where the only way to access new therapies is by paying out-of-pocket for personal importation or through Health Canada's Special Access Program, creating an environment where the community is consistently advocating for treatments to come to Canada.
- **Impact on Timelines:** Once a drug eventually does come to Canada, the proposed evaluation criteria outlined in the draft Guidelines could increased timelines for price reviews. This will delay an already lengthy process even further, making it challenging for people with ALS to access therapies during a timeframe where they will be most effective. When you consider that ALS kills 1,000 Canadians a year, the longer it takes for pharmaceutical manufacturers to apply for approval, begin reimbursement procedures, and market therapies in Canada, thousands of Canadians living with ALS will die before they can access the treatments they need.

HCCC Recommendation #3: *That the Federal Government require PMPRB to hire a third party to conduct a formal assessment of the potential and real-time impacts of the reforms on research investment and activity in Canada (including clinical trials).*

- **Impact on Clinical Trials**: In the face of the terminal diagnosis of ALS, participation in clinical trials can offer people living with ALS access to cutting-edge therapies that could have therapeutic benefits. If manufacturers do not see Canada as a viable market for innovative therapies, they will not be encouraged to invest in Canada. This means people living with ALS will have to advocate for participation in trials in other countries in order to access new therapies. This may also limit the amount of Canadian data available to Health Canada when the manufacturer applies for market approval.

HCCC Recommendation #4: *That the Federal Government require that PMPRB, along with other appropriate agencies, immediately establish a formal mechanism for meaningfully and continuously engaging patient representatives in its decision-making and processes to ensure patient voice, choice and representation.*

- **Impact on Patient Decision-Making:** We want to reiterate that this is a vitally important recommendation. When evaluating therapies for rare diseases, the real impact of the therapy on a patient population must be considered. Moreover, we believe this understanding can only come from meaningful patient engagement where representatives from the community are a part of decision-making. Mechanisms must go beyond processes like the CADTH Patient Input Submission, where patient representation does not get taken into consideration during decision-making. We would, therefore, recommend that patients of various diseases are members of committees and boards that make decisions for new drug prices.

In alignment with HCCC, ALS Canada supports efforts to improve affordable access to therapies. We believe this must be done in a way that ensures Canadians' swift access to innovative treatments and clinical trials of new medicines. Any changes to Canada's drug access pathway – including the PMPRB – must be flexible and responsive to the needs of people living with ALS and encourage more proven therapies to be developed, sold, and reimbursed in Canada.

Should you require any clarification or further information, please don't hesitate to get into contact with me. I urge you to please take into account the needs and perspectives of our community and work with us towards a future without ALS.

Sincerely,

Tammy Moore

CEO