Written Submission to the 2022 Proposed updates to the PMPRB Guidelines

By: ALS Society of Canada

December 2022

Introduction

Our country's healthcare system is a source of national pride for many Canadians. Affordable, equitable and timely access to therapies is something that Canadians expect. While we recognize the proposed updates to the guidelines' goal of lowering pharmaceutical drug prices, we urge you to understand that such changes must be implemented in a comprehensive manner and without creating further barriers to accessing innovative therapies for any Canadians, especially those in the rare disease space.

The ALS Society of Canada, alongside the Health Charities Coalition of Canada (HCCC), have provided input into the PMPRB Modernization process since February 2018. Our message has been consistent and clear – access to innovative therapies is a critical issue for the Canadians we serve, and efforts intended to lower pharmaceutical drug prices must be made in a balanced way that continues to encourage innovation and does not result in the delay of launches and introduction of new medicines in the Canadian market.

Receiving a diagnosis of amyotrophic lateral sclerosis (ALS) is something no person ever wants to hear because it means death within a matter of months or years. 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. 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. With more ALS therapies currently in clinical trials worldwide than ever before, we do not want to see a future where pharmaceutical companies elect not to come to Canada, yet regulatory bodies in other countries are approving therapies. Every Canadian currently living with ALS – and every Canadian diagnosed today, tomorrow and in the future – needs the ability to access innovative therapies as soon as possible. Manufacturers must see Canada as a viable market to run clinical trials and pursue regulatory approval.

Ultimately, we believe an effective and sustainable drug approval process is critical in providing timely access to medicines for Canadians. Any changes to Canada's drug access pathway – including the PMPRB – must be flexible and responsive to the needs of rare diseases, such as ALS, and encourage more proven therapies to be developed, sold, and reimbursed in Canada. It is essential that tomorrow's proven therapies be accessible to all Canadians in a timely, equitable and affordable way.

RECOMMENDATIONS

Recommendation #1: That an independent third party be hired to conduct a formal assessment of the potential and real-time impacts of the proposed guideline changes on access to medicines (including access to clinical trials) in Canada and across therapeutic areas.

ALS research is at a time of unprecedented momentum. There has been more progress in the last 5 to 7 years than in the last century. Many of the top ALS researchers in the world believe the scientific community is now poised to find treatments that can significantly alter the course of the disease.

For people living with ALS, participation in research and clinical trials means being able to access cutting-edge therapies that could have therapeutic benefits. However, without a clear framework on price regulations and demonstrations of how the new system would work may result in a scenario where manufacturers, especially small companies within the rare disease space, may be reluctant to hold clinical trials and bring their therapies to market in Canada. This would not only prevent Canadians living with ALS from having early access to therapies that may benefit them but would also interfere with the work and progress made by the scientific community, including ALS researchers.

Any changes to the guidelines must ensure that it does not create additional barriers or delays in the length of time that it takes for Canadians to access new medicines and should strive to create an environment that makes Canada a country of choice for the industry to bring new therapies, from research and development to clinical trials through to new drug submissions.

Recommendation #2: The PMPRB establishes formal mechanisms for meaningfully and continuously engaging patient representatives in its decision-making and processes to ensure patient voice, choice and representation.

The ALS Society of Canada is pleased to continue to provide feedback as part of the PMPRB's consultation process. However, it is disappointing to see that our and many other patient groups' recommendations are still outstanding and are being repeated for this consultation, especially given the PMPRB website clearly articulates that the Board is *committed to listening to the voices and views of Canadians and including them in decision making.*

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 the decision-making process. Effective and meaningful stakeholder involvement is essential to enable the PMPRB to fulfil its mandate, deliver programs, launch new initiatives, and build public trust.

We would, therefore, request that the PMPRB identify and implement a formal process for patients to be involved in PMPRB policy development and decision-making.

The time is long overdue for PMPRB, along with other appropriate agencies, to establish a formal mechanism for meaningfully and continuously engaging patient representatives in its decision-making and processes. It's time to ensure the patient voice, choice and representation at PMPRB.

Recommendation #3: The implementation of the new guidelines should be paused until the recommendations are properly addressed and patient input is meaningfully integrated into the PMPRB process.

On October 6, PMPRB released its new draft guidelines and announced the launch of a 60-day consultation period with the closure of the consultation on December 5, 2022, to the implementation date of January 1, 2023. It is unclear how feedback will be considered and, where possible, integrated into the guidelines with a tight turnaround time from the closure of the consultation on December 5, 2022, to the implementation date of January 1, 2023.

This short turnaround time to analyze the information and feedback provided does not allow for respectful consideration of stakeholder feedback prior to the implementation date. Furthermore, this tight timeframe leads to the impression that the current consultations are not sincere. Despite consistent feedback to this effect, since the outset, the consultation process has been faulty. It has failed to fully reflect the feedback provided by patients and their representatives, which results in a flawed policy.

The implementation of the new guidelines must be paused until the recommendations are properly addressed and patient input is meaningfully integrated into the PMPRB process.

Recommendation #4: That changes to new investigative criteria will not inadvertently put rare disease patient populations at a disadvantage resulting in further delays in accessing new medicines.

Access to innovative therapies is an urgent issue for people living with ALS. Yet, the current drug approval and reimbursement processes in Canada do not function in a way that reflects the realities of what it means to live with this disease – a swift progressing, fatal disease with no cure.

We understand that the proposed updates to the guidelines around new medicines' intent are to protect the patients by ensuring prices are not excessive. However, we are concerned that for some new medicines, there may not be a therapeutic class comparator price which would increase the likelihood of the product being referred for an investigation. This would not only delay access to therapies for people living with ALS and other rare diseases but would also deter manufacturers from launching drugs for rare diseases in Canada.

For people living with ALS, there is no time to waste. The PMPRB should not be a barrier to accessing future proven treatments. The proposed guidelines should not put any patient populations with few or no therapeutic options currently available at a further disadvantage.

Conclusion

The ALS community measures time not by months or years but by loss – loss of function and loss of life. The impact of an ALS diagnosis on both the person living with the disease and their loved ones is tremendous and pervasive – physically, psychologically and financially. The realities of the disease are harsh – and they reinforce the important role the Government of Canada and the PMPRB play in ensuring people living with ALS have equitable, timely and affordable access to therapies. The ALS community relies on innovative therapies so people living with the disease can live better lives. We hope that any efforts made to reform how patented medicines are priced in Canada balance affordability and accessibility to medications.