RAREi
THE CANADIAN FORUM FOR
RARE DISEASE INNOVATORS

RAREi's Submission to PMPRB on the Guideline Monitoring and Evaluation Plan (GMEP)

June 2021

1. Introduction

The Canadian Forum Rare Disease Innovators (RAREi) appreciates the opportunity to offer input into the Patented Medicine Prices Review Board's (PMPRB's or the board's) proposed Guideline Monitoring and Evaluation Plan (GMEP).

RAREi wants to first emphasize the need to delay the implementation of the new pricing regulations and guidelines. This delay is needed in recognition of the impact of the ongoing COVID-19 pandemic on the entire biopharmaceutical sector and the health system, and the need to reconsider the approach to reforming the PMPRB's pricing framework.

As has been made clear in all RAREi submissions to date, the new pricing regime will have long-standing negative effects on patients, especially those suffering from rare disorders, and put Canada at a competitive disadvantage in terms of medication launches and clinical research. The changes to the pricing regime also need to be further considered to ensure they do not undermine the federal government's efforts to develop a national rare disease treatment strategy. Ultimately, federal pharmaceutical policies should improve and not curtail access to rare disease therapies.

Further, RAREi believe that the board's plans to evaluate the impact of its reforms retrospectively is akin to closing the barn doors after the horse has bolted. Early in the reform process, RAREi along with numerous stakeholders and analysts urged the federal government and the PMPRB to undertake a comprehensive review of the implications of the proposed changes **before** they are implemented. To help address this knowledge gap, RAREi supported the development of case studies to assess how patentees would react in terms of product launch decisions in Canada.[1] There are legitimate concerns about the negative impact of the reforms on access to medicines and the viability and prosperity of Canada's life sciences sector that remain unaddressed to this day.[2]

[1] https://www.canadianhealthpolicy.com/products/new-patented-medicine-regulations-in-canada--updated-case-study---en-fr-.html.

[2] https://macdonaldlaurier.ca/files/pdf/20210525_More_than_a_dose_of_collaboration_Rawson_Adams_PAPER_FWeb.pdf?mc_cid=44923e3a19&mc_eid=UNIQID.

With these initial comments as context, please see below for RAREi's input on the draft GMEP, which it believes would help inform an external and independent review and evaluation of the new PMPRB pricing regime.

2. Feedback on the draft GMEP

An independent third-party evaluator is needed to assess the effects

The fact that the PMPRB intends to evaluate the impact of its own new pricing regime is problematic.

Several specific board initiatives underscore the ongoing bias that precludes the PMPRB from undertaking an evaluation in a neutral, impartial and fair manner, including, but not limited, to the following:

- Inappropriate public and internal-to-government advocacy for the reforms
- Consistent efforts to undermine and ignore legitimate stakeholder concerns
- Published statements reflecting negative attitudes toward its regulated sector
- The February 2021 communications plan designed to counteract what it claims to be "disinformation" by the pharmaceutical industry and patient groups

The federal government needs to commission an independent, impartial, third-party evaluation of these reforms by a respected external organization. This is particularly vital given the controversial nature of the reforms, the sheer number and breadth of parties that have expressed serious concerns with the approach taken and the fact that the vast majority of recommended changes to the approach by stakeholders remain unaddressed.

In sum, RAREi believes that the PMPRB changes need to be independently evaluated so that patentees and Canadians can obtain objective information regarding the effects of the new regulatory regime. The PMPRB and Parliament, to which the board reports, can then take the steps necessary to make adjustments to the regime based on the results of that evaluation.

In this context, RAREi offers the following specific recommendations to be considered by an independent evaluator.

GMEP's scope should be restricted

The draft GMEP is overly broad, and there is no need to assess the entire pharmaceutical policy environment in Canada, including health technology assessments, price negotiations and reimbursement. The plan should be restricted to assessing a smaller number of metrics that broadly cover the effects of the new pricing regime on access to medicines, clinical trials, research and development, employment in the sector and the administrative burden on patentees.

Appropriate baseline for assessment needs to be specified

The draft GMEP does not identify the specific timeframe that will be used as the baseline for the evaluation. In order to appropriately capture the effects of the pricing reforms, RAREi believes the assessment should use the period before December 2017 as a baseline. That is when the first draft amendments to the *Patented Medicines Regulations* were published in *Canada Gazette Part I*. At that time, patentees were made aware of the federal government's approach to reforming the PMPRB's price review regime and started to react to the anticipated changes by altering their decision-making regarding the commercialization of their products in Canada.

Parameters for assessing the impact on access to medicines

The evaluation of the impact of the new pricing regime on access to medicines should look at whether medicines are sold on the Canadian market instead of focusing on their approval status. This is important because some pharmaceutical companies may seek approval of a medicine but decide never to sell it on the Canadian market. Approved medicines that are not marketed in Canada cannot be accessed by Canadian patients.

In addition, given that that the new pricing regime is expected to disproportionately affect rare disease medicines, we recommend that GMEP specifically assess and report on the impact of the changes on access to these therapies. This is especially important given that Canadian rare disease patients already face immense challenges in accessing the treatments they need to get better and survive. There is a need to appropriately assess these effects so that measures can be swiftly adopted to mitigate any harm caused.

Assessing the impact on clinical trials

In terms of tracking clinical trials activity, it is necessary to focus on industry-funded late-phase (Phase III and IV) clinical trials, since these are the ones that have been, and will be, most affected by the reforms. According to recent research, these types of clinical trials, which are far more expensive than those conducted in earlier stages, and which provide patients with early access to promising new therapeutics, have already declined by more than 20% from 2015 to 2020 as a result of the anticipated reforms.[3]

The level of advanced clinical trial activity is also a good gauge to help determine whether Canada is viewed by pharmaceutical companies as a viable first-tier launch market. Given the ethical requirement of ensuring that patients who are well stabilized on a trial medication can continue to benefit from it after the completion of the trial, innovators will not conduct advanced clinical trials in Canada if they do not intend to market their products in this country.

Formula used for tracking research and development investments

RAREi is encouraged by the intention to take account of research and development (R&D) investments that go beyond what is eligible for Scientific Research and Experimental Development (SR&ED) credits.

[3] https://www.canadianhealthpolicy.com/products/clinical-trials-in-canada--worrying-signs-remain-despite-pmprb---s-superficial-response.html.

The pharmaceutical industry has been calling on the board to change its outdated and restrictive R&D spending formula. The results from its reliance on that inappropriate formula have been used consistently to criticize the industry for not living up to its investment commitments. Evaluations of industry investments that rely on a broader definition of R&D will help better capture the extent of industry investments and provide more of an apples-to-apples comparison when benchmarking against international data.

RAREi recommends, however, that the board makes sure to apply the same formula to determine the level R&D investments prior to and post reforms. There is a need to ensure that one is comparing the same type of investments so that the effects of the pricing reforms can be properly assessed.

Reporting on medication spending

RAREi does not believe that medication spending should be assessed as part of the GMEP. Such spending is not only affected by product prices, but it varies depending on the use and availability of medicines. As such, RAREi does not view this as a relevant benchmark for assessing the impact of the pricing reforms. Nevertheless, RAREi would still like to offer a few comments regarding how the PMPRB has been reporting on medication spending.

Recent PMPRB communications have emphasized increases in medication spending generally and, in particular, the growth in the impact of high-cost treatments. However, the board has failed to contextualize these trends by looking at medication spending in the context of overall health care spending and economic growth. If it had, it would have noted that the share of national health care spending represented by patented medicines was almost the same in 2019 as in 2000 (6.4%) and their share of gross domestic product (GDP) was approximately the same in 2019 (0.7%) as in 2003 (0.8%). This is a remarkable period of near zero average annual relative expenditure growth. Even at manufacturer 'list' prices, which do not represent the actual price paid in most cases, patented medicines expenditure represents a small fraction (6.5% in 2019) of national health expenditure. They have never excessed 8% of national health care spending. As a recent Canadian Health Policy Institute report noted, federal government and PMPRB claims that "excessive" prices for patented medicines are creating a health care sustainability crisis do not stand up to objective scrutiny.[4]

The trends in public drug plan spending are even more notable. During the past 10 years, medication spending growth by provincial and territorial (P/T) governments has been lower than their collective health spending growth in nine of those years. In 2019-20, total P/T medication spending was estimated to represent 7.2% of their total collective health spending, the lowest proportion since 1999-2000.[5] These data demonstrate the importance of contextualizing medication spending in order to avoid misleading the public and policy makers into thinking that these costs are out of control.

Another important point to be make is that the PMPRB tends to report on overall medication spending when its mandate is limited to patented medicines. It would be more relevant for the

[4] https://www.canadianhealthpolicy.com/products/patented-medicines-expenditure-in-canada-1990-2019.html.
[5] https://www.cihi.ca/sites/default/files/document/nhex-trends-narrative-report-2019-en-web.pdf.

PMPRB to report on costs of patented medicines, which only represent a fraction of total medication spending.

3. Concluding remarks

In closing, RAREi would like to reiterate its request that the PMPRB regulations and guidelines be paused to enable the industry to continue to focus on responding to the pandemic and to allow the government to revisit its approach.

The PMPRB and the government should be focused on ensuring it is putting in place a clear, predictable and well-balanced pricing regime rather than creating a self-monitoring mechanism to assess the effects of a flawed reform. After the new pricing regime is implemented and has reduced patient access to medicines and research investments, it will be too late to remediate the damage suffered by rare disease patients.

RAREi believe that challenges regarding the accessibility and affordability of rare disease medicines can be resolved through innovative negotiated solutions and as part of the federal government's national rare disease treatment strategy.