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Patented Medicine Prices Review Board Changes and Their Impact on Canadians

IMPACT REPORT

December 2020 // **Gastrointestinal Society**

Contents

Update

On December 30, 2020, Health Canada postponed the implementation date of the new PMPRB changes from January 1, 2021 to July 1, 2021 in recognition of the challenges brought by the COVID-19 pandemic. Details will be published in the *Canada Gazette, Part II* on January 20, 2021. Contact the federal government now to share how important access to new and lifesaving medicines are for you, your loved ones, and fellow Canadians at www.fightforourlives.ca.

Executive Summary

Canada is the only country in the world with a regulator that caps patented medicine prices, but it claims to adopt best practices used in other developed countries by considering value and affordability. It does so under the Patented Medicine Prices Review Board (PMPRB). After the PMPRB sets a maximum price, Canada and its provinces and territories also have other bodies that further negotiate the price of a medicine lower.

Why does this matter to Canadians now?

The PMPRB has been around since 1987, with guidelines as to how it calculates a maximum price. In June 2016 it announced plans to change the guidelines. However, release of the final PMPRB Modernization Guidelines on October 23, 2020 has not given Canadians enough time to **respond to the changes before the January 1, 2021** implementation date.

Experts in the Canadian healthcare system, including patient groups, say this "modernization" could seriously affect access to lifesaving and life-altering drugs. When the amount paid to the manufacturer for publicly-funded medications becomes so low, it might no longer be viable for the companies to sell their medicines in Canada. This includes both new drugs and existing drugs, which could be pulled from the Canadian market.

In the full report we explore the changes to the guidelines, provide background, and explain the potential effects of the changes. We hope you take the time to read about them now, before it's too late. We identify the ways in which healthcare systems interact with our lives, as we use prescription medicines, medical devices, participate in clinical trials, and access patient support programs. We also provide real world examples to show the effects of these policies on Canadians today.

In addition to having a significant impact on price, the PMPRB guideline changes are generating a high level of uncertainty, which could jeopardize the launch of new medicines by significantly decreasing Canada's attractiveness as a priority jurisdiction in which to seek regulatory approval to sell their new medicines. Pricing uncertainty will impact drug manufacturers, but this concern reaches right down to the end user of medications. We foresee significant delays for Canadians to access important new therapies that are lifesaving or life-altering. We see this affecting patient support programs and clinical trials. A decision not to launch a product in Canada would eliminate access for all patients, even those who have private insurance for drugs.

The PMPRB is also executing this regulatory overhaul as our healthcare systems continue to prioritize responding to the challenges of the COVID-19 pandemic. This is not the time!

What can you do?

The Canadian Organization for Rare Disorders (CORD), of which we are a member, has launched a movement, **www.fightforourlives.ca**,[1] to call on the federal government to stop the PMPRB guidelines coming into effect.

Individuals across Canada have taken part in their letter-writing campaign, and you can too. Share your story on the importance of access to medicines for you, your loved ones, and fellow Canadians. You do not need to have a rare disease or disorder, or chronic health conditions to participate. You can also contact us at the Gastrointestinal Society to learn more of how we can ensure the right people hear your voice.

Background

What is the PMPRB?

The Patented Medicine Prices Review Board (PMPRB)[2] is a federal agency established as an integral part of the 1987 amendments to the Canadian *Patent Act*.

Vision: A sustainable pharmaceutical system where payers have the information they need to make smart reimbursement choices and Canadians have access to patented drugs at affordable prices.

Mission: A respected public agency that makes a unique and valued contribution to sustainable spending on pharmaceuticals in Canada by providing stakeholders with price, cost, and utilization information to help them make timely and knowledgeable drug pricing, purchasing, and reimbursement decisions and acting as an effective check on the patent rights of pharmaceutical manufacturers through the responsible and efficient use of its consumer protection powers.

Determining Price

The *Patent Act* identifies factors for the PMPRB to consider in determining whether the price of a patented medicine is excessive. These factors include:

- the prices at which the medicine has been sold
- the prices at which other medicines in the same therapeutic class have been sold in the relevant market
- the prices at which the medicine and other medicines in the same therapeutic class have been sold in countries other than Canada
- changes in the Consumer Price Index
- such other factors as may be specified in any regulations made for the purposes of subsection 85(1) of the *Act*

In practical terms, the PMPRB sets and monitors the list of transparent prices of patented medicines to ensure Canadian prices are deemed 'non-excessive' compared with a group of other countries.

The Drug Pricing World Has Changed Since 1987

Here are some of the measures the public agencies are taking to further reduce and manage drug costs:

- introduction of health technology assessment (HTA) – Common Drug Review and pan-Canadian Oncology Drug Review (pCODR), plus provincial reviews
- price freezes by provinces
- expansion of mandatory generic substitution
- newer reimbursement policies such as reference-based pricing, maximum allowable costs, lowest cost alternative, therapeutic substitution
- introduction of listing agreements, confidential rebates, risk-sharing
- pan-Canadian Pharmaceutical Alliance (pCPA), which allows for joint negotiation on product listing agreements and generic price reductions
- the introduction in 2019, of the Canadian Drug Agency, a new national drug agency that would build on existing provincial and territorial successes, and take a coordinated approach to assessing effectiveness and negotiating prescription drug prices on behalf of Canadians as part of a national pharmacare program

Changes

So why make changes now?

On October 23, 2020, the Government of Canada released the final PMPRB Guidelines.[3] Our analyses in this paper are based, for the most part, on a version released in June 2020, as we began our research months prior to the October publication. Our feedback to the PMPRB consultations, submitted on August 4, 2020, is available on our website at www.badgut.org/pmprb-input-2020/. Several components of the PMPRB's modernization process continue to be under review so this document might be subject to change.

Changes

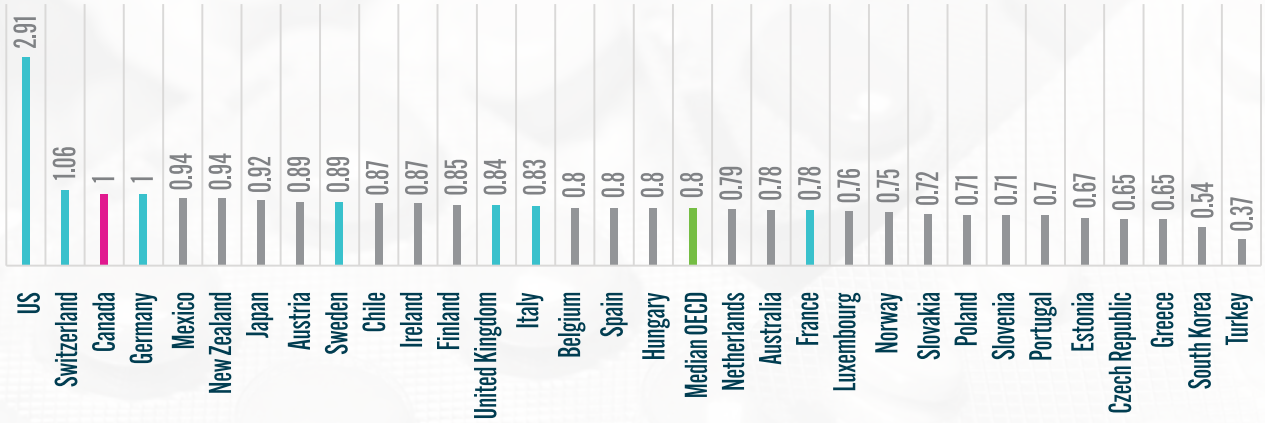
Drastic and complicated changes to the way drug prices are set in Canada will be coming into effect on January 1, 2021. These changes could have significant negative impacts on current and future health outcomes for Canadians. Here are some of the changes:[3]

- the PMPRB's initial mandate was to ensure that list prices are not excessive, but this mandate has expanded to address net prices using new, economics-based price regulatory factors to ensure prices reflect value and Canada's willingness/ability to pay in addition to those that payers already actively use
- update the list of countries used for international price comparisons to align better with its consumer protection mandate, but its effect is really to keep Canada in the middle of OECD countries
- reduce reporting obligations for patented veterinary, over the counter, and generic medicines
- set out new information reporting requirements to operationalize the new price regulatory factors
- require patentees to report price and revenue information that is net of all domestic price adjustments[4]
- compares the price to the median price set in a basket of 7 comparator countries: France, Germany, Italy, Sweden, Switzerland, the UK, and the US (countries in red dropped for new basket) and the changes to this basket of 11 countries: Australia, Belgium, France, Germany, Italy, Japan, the Netherlands, Norway, Spain, Sweden, and the United Kingdom (countries in green added for new basket)
- with the introduction of the Maximum Rebated Price (MRP), the PMPRB is also regulating the negotiated price ceiling, driving prices to unsustainable levels (this process is on hold due to court challenges)
- implementation date moved from January 1, 2020 to July 1, 2020 to January 1, 2021

Prices set by PMPRB are the retail public price that typically are then subject to confidential negotiations between manufacturers and public and private drug plans, which lowers the prices even further, sometimes pushed to a point where it is no longer sustainable to provide the product to the Canadian market.

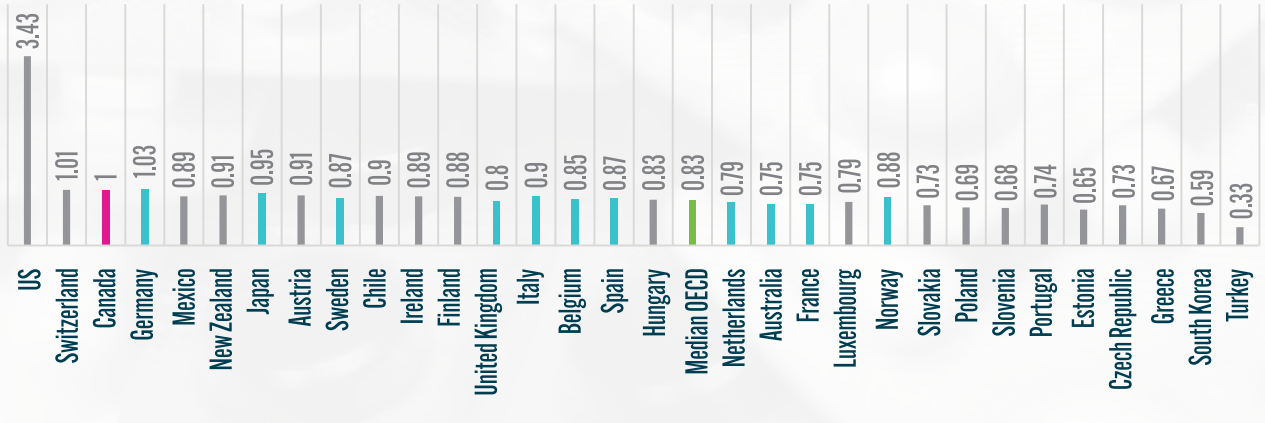
Now, many have questioned the continued relevance of the PMPRB. In sum, PMPRB's sweeping and complex changes to the methodology used to set net prices for medicines in Canada will all play a role in determining both the initial list price and the subsequent prices for the same medicine moving forward. This significantly raises the uncertainty for the pharmaceutical industry to plan and for physicians and patients to rely on a medicine supply.[5]

Average Foreign to Canadian Price Ratios, Patented Drugs, OECD, 2016



PMPRB7 (previous comparator countries); listed in descending order

Average Foreign to Canadian Price Ratios, Patented Drugs, OECD, 2018



PMPRB11 (new comparator countries)

Discussion

PMPRB Claims

In its *Amendments to the Patented Medicines Regulations – Questions and Answers,* the PMPRB claims: [6]

- Canadian prices are higher than most of the seven countries in the current comparator basket, which includes the United States, Switzerland, Germany, Sweden, United Kingdom, Italy, and France
- our prices are also higher than other countries not considered, such as New Zealand, and many EU countries
- US is not an appropriate comparator country
- patent-holders have not lived up to their 10% research and development (R&D) commitment
- when we look at all drugs, the Organisation for Economic Co-operation and Development (OECD) reports Canada has the second highest expenditures per capita, at $958 in 2012

Importantly, stakeholders dispute some of these claims made by the PMPRB, and in comparison to other countries, Canadian prices did decrease from 2016[7] to 2018[8].

How exactly will PMPRB be reviewing drug prices?

If you want to dig into the incredibly complex formula the PMPRB is proposing, refer to Section V of the PMPRB final Guidelines.[3]

- when a new drug enters the Canadian market, PMPRB will categorize the product into one of the following:
  1. grandfathered patented medicines
  2. line extensions
  3. gap medicines
  4. new patented medicines
- each category has its own processes for determining the price ceiling of that specific product and unique price review factors
- for grandfathered patented medicines, PMPRB will assess the drug with a variety of other factors
- for all other categories, the price review factors are similar to grandfathered patented medicines, with the addition of three new factors:
  - pharmacoeconomic value
  - market size
  - gross domestic product (GDP) and GDP per capita

Critics Warn that Changes Have Risks

Stakeholders throughout the healthcare systems are speaking out about these changes, and they suggest that:[9]

- the PMPRB guidelines do not take into consideration other aspects of the Canadian system
- one study concluded that the new rules would require a >50% decrease in the manufacturer's price versus the current rules (PMPRB's initial proposal was an excessive 80-90% decrease, so we are grateful they reduced it, but they have not reduced this requirement enough, as a price decrease of even this magnitude will impact patient access to medications)
- an objective analysis of the PMPRB's own data strongly suggests that any regulatory change that ends up arbitrarily depressing prices runs the risk of reducing the availability of new medicines for Canadian patients

How does the PMPRB fit within the medicines review process in Canada?

At the time of its inception in 1987, the PMPRB was the only government body that controlled and monitored the prices for patented medicines. Upon receiving a license to sell a medicine in Canada, the patent-holder applied to the PMPRB and negotiated a list price for the Canadian marketplace. That process remained the same for the first 30 years or so.

Over time, the PMPRB process and the resultant negotiated list price has become less and less relevant to Canadian patients. Bodies such as the Canadian Coordinating Office for Health Technology Assessment (CCOHTA) in the early 1990s, which morphed into the Canadian Agency for Drugs and Technologies in Health[10] (CADTH) in 2006, the pan-Canadian Pharmaceutical Alliance[11] (pCPA) in 2010, and each provincial and territorial, and some federal jurisdictions that also negotiate prices, have undertaken much more rigorous clinical and health economic evaluations of medicines and had a more direct impact on the actual price that patients, private insurers, and governments pay.

The *Institut national d'excellence en santé et en services sociaux* (INESSS)[12] was created on January 19, 2011 from the merging of the *Conseil du médicament* and the *Agence d'évaluation des technologies et des modes d'intervention en santé* (AETMIS) to serve Quebec.

PMPRB changes also do not include opportunities for clinicians and patients to provide input in the drug review process. This is a departure from international and domestic best practices on the review of medicines. Currently, Canadians can submit feedback to CADTH in their review of drugs, which CADTH incorporates into their final recommendations to provinces. It is a well-regarded process and members of CADTH have continuously expressed the value of patient input in their clinical reviews. A similar process occurs in INESSS for Quebec. Provinces and territories have formal or informal patient input opportunities, but PMPRB does not.

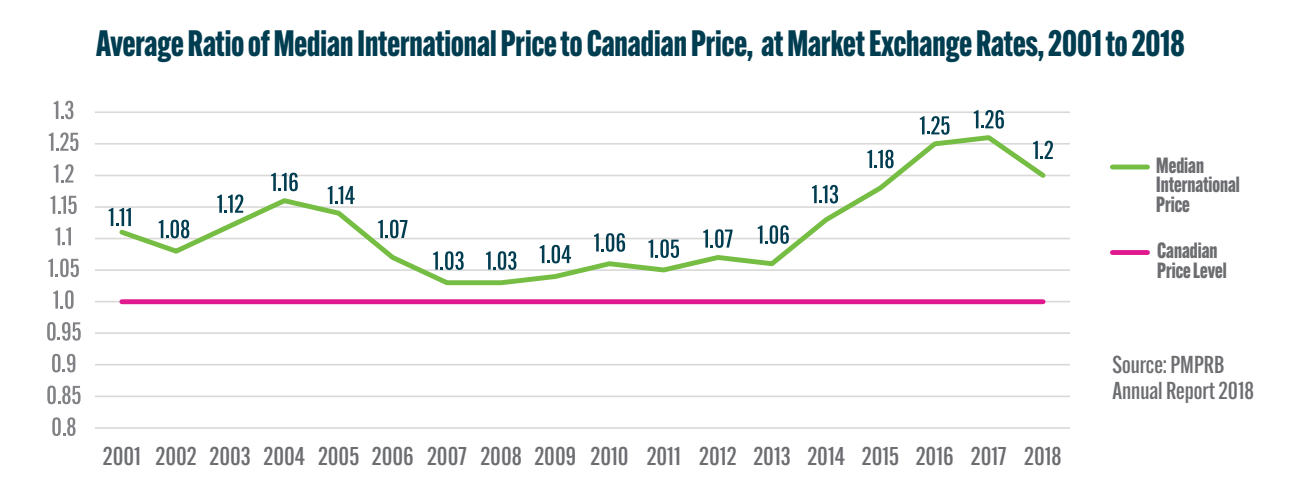
How much do Canadians spend on medicines compared to other parts of the healthcare system?

According to the Canadian Health Policy Institute[13] (CHPI), the PMPRB published figures show that in 2017, total sales of patented medicines in Canada at manufacturer list prices before rebates totaled $16.8 billion. Analysis showed that gross sales of patented drugs have accounted for less than 1% of gross domestic product (GDP) for the last 28 years. Patented drug sales were the same percentage of GDP in 2017 (0.78%) as in 2003 (0.78%), a 15-year period of zero average annual growth relative to GDP. Accounting for public drug plan rebates, the net national cost of patented drugs was only 0.68% of GDP in 2017.

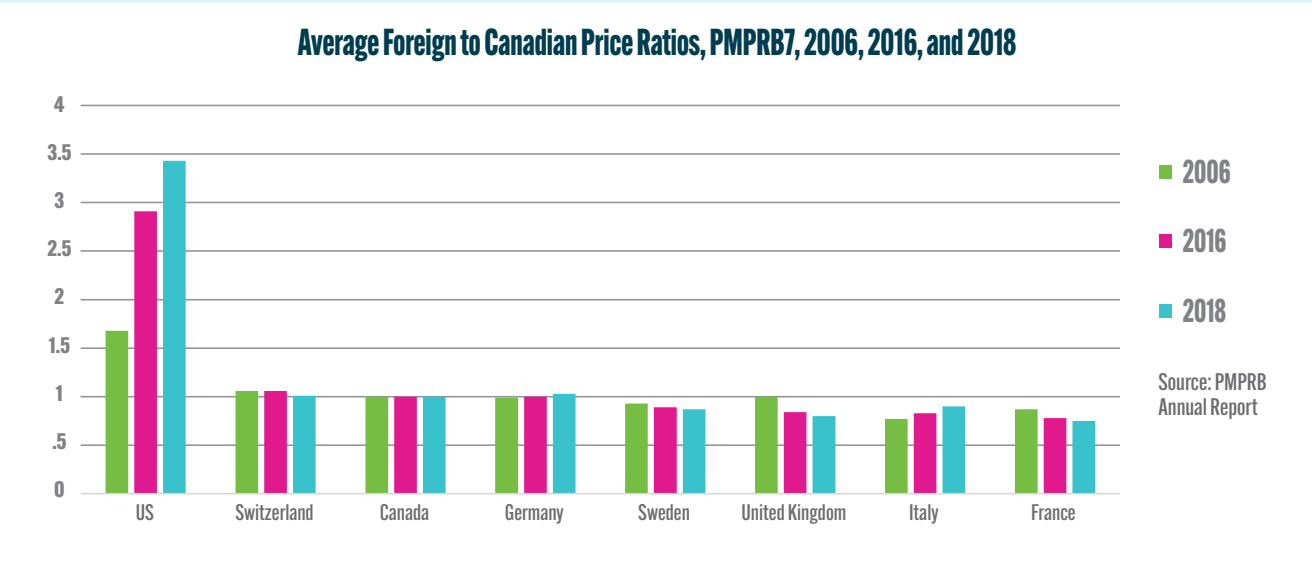
Gross sales of patented drugs accounted for 6.9% of the $243.4 billion reported by the Canadian Institute for Health Information (CIHI) for total health spending in Canada in 2017. Patented drugs accounted for a smaller percentage of total health spending in 2017 (6.9%) than in the year 2001 (7.1%), a 17-year period of near zero average annual relative cost growth. Accounting for public drug plan rebates, the net national cost of patented drugs was only 6.0% of total health spending in 2017. (see Appendix B)

How have pharmaceutical prices in Canada compared to other countries?

From PMPRB published data, Canadian drug list prices are consistently and significantly *below* the Median International list prices for countries currently monitored by PMPRB.



While Sweden, Italy, UK, and France have prices below Canada in 2018, the difference between these range from 0.1 to 0.25 at most. In the span of more than a decade, prices in three of the PMPRB7 (United States, Switzerland, Germany, Sweden, United Kingdom, Italy, and France) have increased while Canada stayed the same.



The guidelines also differ from international practice, where the Organisation for Economic Co-operation and Development (OECD) countries primarily establish reimbursement rates using internal and/or external reference pricing. If they use cost-effective analyses, they incorporate sliding thresholds (often for drugs for rare diseases/disorders) and may use these to determine whether to cover a drug, rather than establishing its price. Drug regulation in OECD countries such as Australia, France, Germany, Norway, Switzerland, and the United Kingdom are also national, and they lead to uniform prices and reimbursement. This is contrary to Canada's complex medicine review process, as described above.[14]

Aren't lower drug prices a good thing?

Clearly, patients, employers, government payers, and insurance companies would welcome measures that lower drug prices in Canada. However, there are a few things to keep in mind. The PMPRB primarily regulates patented, sometimes referred to as 'innovative' medicines. These are the latest discoveries brought forward by pharmaceutical and bio-pharmaceutical companies both large and small. The PMPRB is in place to mitigate patented drug prices only and it does not regulate generic prices. While these new guidelines will not directly reduce generic prices, it makes sense that as patented drug prices go down, the price of each associated generic drug will also, eventually decrease. This could lead to generic manufacturers stopping their supply to Canada, as government sets their drug prices as a percentage of the patented drugs. If drastic cuts apply to patented drugs, then the cascade to generic drugs could leave Canadians without generic medicines as well. Currently, Canadians pay some of the highest generic prices in the world.

The reality in healthcare is that the price a company charges for its treatment has a direct impact on every aspect of healthcare, including, its ability to create the next new medicine, its ability to conduct clinical research, its ability to provide patient supports, its ability to employ Canadians, and its ability to budget over the patent lifespan. This should concern all of us.

Any policy changes that dramatically impact patented/innovative new and future medicines must carefully consider the potential for unintended consequences and collateral damage. With these changes, we are genuinely concerned that there will be little incentive to bring new medicines to Canada and that the supply of medicines we do have will dwindle away.

What could this mean for new drug launches?

Innovative Medicines Canada (IMC) is a national membership of pharmaceutical companies and they reported that their members have already experienced job losses, delays in launching new drugs, and members putting their Canadian investments on hold. A November 2019 survey[15] of pharmaceutical and other life sciences leaders by Life Sciences Ontario reported the following impacts:

- virtually every executive expects PMPRB changes to result in 'no launch' decisions and delayed launches in Canada
- they expect oncology, followed by biologics, rare disease, rheumatology, and gene therapy to be most affected by the changes
- 94% of companies reported negative impacts to clinical trial research
- 96% of those surveyed expect a negative impact in employment in Canada

IMC submission cites concerns that "most new drugs would trigger the 50% price reduction standard" described below and predicts that "it will lead to frequent disputes" further delaying access and disincentivizing manufacturers to launch in Canada.[16]

What other impacts will there be if PMPRB implements these changes?

There will be unintended consequences associated with these changes, all of which will have a negative impact on healthcare delivery, in general, and patients specifically. Here is a partial list and we will look more carefully at each one:

The PMPRB changes will significantly reduce:

- the number of new, innovative medicines that manufacturers launch in Canada
- the number and size of clinical trials that take place in Canada
- Canadian access to the latest innovations that are part of those clinical trials
- patient support programs and services that pharmaceutical companies currently supply

How will this affect new product launches?

Pharmaceutical companies prioritize marketing their products in the countries that have predictable drug pricing policies. They group countries into what they call Tier I, Tier II and Tier III categories. Pharmaceutical companies decide which countries fall into each tier based on a variety of considerations. For instance, companies often list countries with competitive pricing in Tier I because this means that the country's market is favourable for a successful launch of innovative products. On the other hand, companies launch their products in lower tier countries years later, if at all.

It is most desirable to be among Tier I countries, the first in the world to have new innovative medicines available to our population. Although pharmaceutical companies have been considering Canada a Tier I country, with the PMPRB changes, this ranking is at risk and the consequences of moving from Tier I are grave.

"We are extremely concerned that access to important new therapies will be significantly delayed or denied and that the consequential manufacturers' decisions to not launch products in Canada would eliminate access for all patients," said Gail Attara, President and CEO of the Gastrointestinal Society, "there is a real human cost to delaying and barring access to new medicines."[17]

Price Linked to New Drug Launches in 31 OECD Countries

A 2018 study[18] examined data from the PMPRB and the Organisation for Economic Co-operation and Development (OECD) evaluating the statistical relationship between the number of new drug launches across 31 OECD countries and three independent variables:

- market price level for patented drugs
- per capita gross domestic product (GDP)
- total market size (population) in each country

Results showed that market price level was the only one of the three independent variables that was a statistically significant predictor of the number of new drug launches. The analysis confirms that lower priced markets experienced fewer new drug launches, and vice versa, that higher priced markets tended to experience more new drug launches.

Unfortunately, many organizations predict that the changes to PMPRB will result in significant delays in launching new products in Canada.

According to the Canadian Organization for Rare Disorders (CORD),[19] "The threat of draconian price restrictions has already stopped some new treatments from coming to Canada. In the past year, companies have delayed or withdrawn submissions for more than a half-dozen innovative therapies and an equal number of submissions for extended or pediatric indications have been quietly cancelled." (see Appendix C)

How will these changes affect clinical trial participation?

Participation in clinical trial activities provides Canadians access to the latest, newest, and most innovative therapies within any given disease area. These trials represent hope for the future to conquer debilitating and potentially life-threatening illnesses. Again, if pharmaceutical companies do not consider Canada a Tier I product launch country due to PMPRB reforms, they are very unlikely to conduct the clinical trials needed to achieve licensure in Canada.

Launch priority is a key determinant of discovery and clinical research. It makes sense that manufacturers are reluctant to conduct clinical trials in a specific country unless they are confident that they will be able to commercialize the drug in that country. Currently, Canadians benefit from a robust clinical trial environment, which links patient suffering with various diseases to the latest innovations in treatment. The PMPRB changes as written will significantly reduce clinical trial activity in Canada and therefore deprive Canadians of better treatments for their individual needs as others around the world enjoy these benefits.

According to the World Medical Association, Declaration of Helsinki – *Ethical Principles For Medical Research Involving Human Subjects*:[20] "At the conclusion of the study, every patient entered into the study should be assured of access to the best proven prophylactic, diagnostic, and therapeutic methods identified by the study."

Therefore, manufacturers are ethically bound to provide study patients with the study drug (if effective) at no cost until reimbursement is available. Accordingly, manufacturers are less likely to conduct clinical trials in countries where reimbursement is unlikely and there is sufficient uncertainty as to whether they will achieve an acceptable price or reimbursement level.

Again, according to CORD, "The inability to access clinical trials is literally a death sentence for patients with life-threatening or progressive diseases with no other effective treatments. Clinical trials have also helped to prepare clinicians and facilities to administer these therapies as soon as they are approved, saving many more lives."[19]

How will changes affect patient support and compassionate access programs?

With fewer product launches and fewer clinical trials in Canada, another inevitable consequence of the stated PMPRB reforms will be a dramatic reduction in patient support initiatives. According to a recent survey published by Life Sciences Ontario,[21] 73% of Canadian pharmaceutical executives predicted a 'somewhat negative impact' or 'significant negative impact' to patient support programming. In the same survey, 70% of executives predict negative impacts on availability of medicines through compassionate access programs, in which they provide medicines at no charge.

How will this affect my patient support program?

With the PMPRB changes, we expect dramatic reductions in patient support materials, patient support programs, and compassionate medication releases. Patient support programs typically provide, among other things:

- specialty pharmacy services
- infusion services (if applicable)
- nurse support and self-care training (e.g., subcutaneous injections)
- reimbursement coordination
- co-pay assistance
- compassionate access for patients with no drug insurance coverage

Do we really need the PMPRB?

In 2017, CHPI[22] published a detailed critique of the original PMPRB proposal. The paper evaluated the merits of the proposed economics-based price regulation factors and pharmacoeconomic evaluation, identifying significant problems and limitations not acknowledged by the PMPRB. It also examined the relevancy of the PMPRB, concluding that, at best, its mandate is redundant and, at worst, obsolete.

Analysis of the PMPRB published data[23] shows that there is no health-spending crisis related to the prices of patented medicines that justifies new regulation. In fact, the weight of available evidence suggests the guidelines could lead to higher total healthcare costs by hindering clinical access to efficient innovative pharmaceutical treatment technologies. Furthermore, a detailed examination of the regulations found the proposed pricing rules to be arbitrary and based on questionable assumptions; and raised serious technical concerns about the proposed methods for determining price ceilings.

"The cystic fibrosis (CF) community is the first to experience how these changes will limit access to new, life-changing medicines," said Kelly Grover, President and CEO of Cystic Fibrosis Canada, "but all Canadians are right to be worried." Fighting Blindness Canada President and CEO, Doug Earle, shares the same fears, "We have seen what the CF community has experienced… and we are concerned that we won't be able to access the new medications, gene, and cell therapies that restore sight and avoid blindness."[24]

The PMPRB announced the implementation of the Guidelines Modernization and Evaluation Process (GMEP) to ensure that their proposed changes will achieve its objectives of lowering drug prices while maintaining access to innovative medicines in Canada. However, they have given little information on how it will operate. They originally planned to release the GMEP in early 2020 but, in a recent webinar,[25] they announced that it will not be available until after the finalization of the Guidelines. PMPRB Staff also acknowledged that they "will work things out," giving rise to unpredictability and risks for both pharmaceutical manufacturers and Canadians. However, in response to feedback the PMPRB received to the June 2020 Guidelines, they announced that they will launch consultations with stakeholders on the GMEP.[26]

Court Challenges Against the PMPRB

Pharmaceutical companies have pursued litigations against PMPRB's proposed Guidelines, with one currently on appeal from a recent Federal Court of Canada decision on June 29, 2020. The other case is a constitutional challenge filed in the Quebec Superior Court and its outcome is pending as of November 9, 2020.[27]

In response to the Federal Court decision, PMPRB has revised a few elements of their pricing review process. However, they may be changed depending on the appeal court decision.[26]

What about COVID-19?

In a May 12, 2020 article in the Financial Post,[28] the authors warn Canadians that PMPRB changes are likely to prevent early access for Canadians to a new COVID-19 vaccine and state, "This uncertainty may be a major disincentive to bringing a new vaccine to Canada." They also question whether a manufacturer will want to bring a COVID-19 vaccine to Canada if the PMPRB can subsequently reduce its tendered price.

The Vaccine Industry Committee of BIOTECanada, the national association of biotechnology companies, wrote that, "If a new vaccine emerged (e.g., coronavirus vaccine) within a global public health threat, the uniquely Canadian requirements, as proposed in the draft guidelines, would negatively affect a potential new vaccine candidate and could result in issues of access and supply."

Even the Public Health Agency of Canada's Centre for Immunization and Respiratory Infectious Diseases is concerned about vaccines coming under the PMPRB's price guidelines.

Remarkably, on August 31, 2020, Janssen Inc. announced an agreement in principle to supply the Government of Canada with a COVID-19 vaccine under which the Government of Canada would purchase up to 38 million doses of Janssen's *Ad26.COV2.S* vaccine candidate on a **not-for-profit basis** for emergency pandemic use.[29] There are also similar deals with Novavax, Moderna, and Pfizer. Others could follow.

On September 17, 2020, the PMPRB announced that there will be a special regulatory pathway for patented medicines that appear on the *List of Drugs for Exceptional Importation and Sale* and those listed on the *Interim Order Respecting the Importation, Sale and Advertising of Drugs for Use in Relation to COVID-19*.[26] PMPRB will not review or investigate medicines on these lists, unless a Minister of Health (federal, provincial, or territorial) files a complaint. However, price reviews will apply if either the Interim Orders expire, are revoked, or on a patented medicine that is removed from the list.

What can you do?

It is crucial that we raise awareness of these impending changes that will limit our access to innovative and lifesaving medicines. Join us in our advocacy to call on the federal government to postpone the implementation of the final PMPRB Guidelines.

The Canadian Organization for Rare Disorders (CORD), of which we are a member, has launched a movement, **www.fightforourlives.ca**, to call on the federal government to stop the PMPRB guidelines coming into effect.[1] Individuals across Canada have taken part in their letter-writing campaign, and you can too. Share your story on the importance of access to medicines for you, your loved ones, and fellow Canadians. You do not need to have a rare disease or disorder, or chronic health conditions to participate. You can also contact us at the Gastrointestinal Society to learn more of how we can ensure the right people hear your voice.

Conclusions

In addition to having a significant impact on price, the PMPRB guideline changes are generating a high level of uncertainty, which could jeopardize the launch of new medicines by significantly decreasing Canada's attractiveness as a priority jurisdiction in which to seek regulatory approval to sell their new medicines. The changes can also affect existing medications for which prices go so low that, suddenly, they are no longer available to Canadians. Pricing uncertainty will impact drug manufacturers, but this concern reaches right down to the end user of medications. We foresee significant delays for Canadians to access important new therapies that are lifesaving or life-altering. We see this affecting patient support programs and clinical trials. A decision not to launch a product in Canada would eliminate access for all patients, even those who have private insurance for drugs.

The PMPRB is also executing this regulatory overhaul as our healthcare systems continue to prioritize responding to the challenges of the COVID-19 pandemic. This is not the time!

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Gastrointestinal Society's Mission

We're committed to improving the lives of people with gastrointestinal and liver conditions, supporting research, advocating for appropriate patient access to healthcare, and promoting gastrointestinal and liver health.

Registered Charity Number 817065352RR0001

Appendix A

What does the PMPRB do?

The Canadian Government, in 1987, gave the PMPRB the mandate to regulate the prices of patented medicines sold in Canada and to ensure that prices were not excessive. Here are a few more details:

- operates as an independent quasi-judicial federal body under the *Patent Act*
- dual mandate: Regulatory, to ensure that prices charged by patentees for patented medicines sold in Canada are not excessive, and Reporting, to report on pharmaceutical trends of all medicines and on research
- determines maximum non-excessive price a patent-holder can sell in Canada; maximum average potential price (MAPP)
- encouraging innovation and protecting consumers against excessive pricing by monopoly providers of patented products

PMPRB Achievements

- since 1987, PMPRB has achieved its purpose:
    - stopped the trend for prices to increase more than the consumer price index
    - lowered prices relative to other countries from 23% above the median in 1987 to below the median today
    - voluntary compliance policy has succeeded – very few cases have progressed to legal proceedings
- 90+ voluntary compliance undertakings (VCUs), usually with payments, to date
- collected $2.8 million in excess revenues in 2014; collected an astounding $35 million in 2017, and yet in 2018 it drastically went down to $315,000 (from PMPRB Annual Reports)

PMPRB's Relationship to Payers

- PMPRB is an independent regulator, not a payer
- federal/provincial/territorial (F/P/T) health ministries are statutory stakeholders
- F/P/T must be consulted on policy
- F/P/T have right to intervene in hearings
- PMPRB sits as an observer on many F/P/T committees, including Canadian Agency for Drugs and Technologies in Health (CADTH) committees
- no involvement with the pan-Canadian Pharmaceutical Alliance (pCPA)
- PMPRB is now a member of the pharmaceutical pricing and reimbursement information (PPRI) which is a network of competent authorities for pricing and reimbursement, as well as to the activities done by Gesundheit Österreich GmbH (GÖG, Austrian National Public Health Institute), which maintains the PPRI network and serves as PPRI secretariat

Appendix B

Health Spending

The shifting shares of health spending in Canada:[30]

- **Hospitals** spending share decreased from 44.7% of total health expenditure in mid-1970s to 26.6% in 2019
- **Physicians** spending as percentage of total health expenditure started edging down in 1988, reversing in the mid-2000s; since 2005, physicians spending has increased, due in part, to the number of physicians, leveling out in 2019 to 15.1%, comparable to the late 1980s
- **Drugs** spending share has been increasing since the mid-1980s, accounting for the second-largest share (15.3% in 2019) after hospitals since 1997

Appendix C

Cystic Fibrosis Example

According to Cystic Fibrosis Canada, cystic fibrosis (CF) is a rare disease affecting approximately 4,200 Canadians, or roughly 1 in 3,600 live births.[31] The first treatment, Kalydeco® (ivacaftor) for CF, a genetic progressive lung disease that is usually fatal to children by the time they reach their teens and early adulthood, was approved in Canada in 2012. However, it is effective for only the 3-4% of the CF population who have a specific genetic mutation.[32,33]

Improved therapies have been developed and two of these have been approved in Canada (with limited public funding for one therapy). However, the manufacturer of the most important new combination therapy Trikafta® (elexacaftor/tezacaftor/ivacaftor) that could be effective for up to 80% of CF patients has concluded that it cannot afford to submit the drug for approval in Canada in the immediate future.[34]

A 23-year-old Halifax woman with CF, Chantelle Lindsay, died on February 19, 2020, after not being able to access Trikafta®, which her medical team said was her final hope.[35] "The federal drug price controls are a death sentence for many patients," said Chris McLeod, Chair of the Canadian Cystic Fibrosis Treatment Society and a CF patient who credits Kalydeco® for his being alive and fully employed today. "The tragic recent passing of Chantelle Lindsay should be a wake-up call for politicians. Canadians are dying because new life-saving medicines are not coming."[36]

Further the Discussion

For more information on PMPRB and what their changes may mean for you and Canada's healthcare system, check out these links.

Evidence that regulating pharmaceutical prices negatively affects R&D and access to new medicines

Yanick Labrie, MSc, Canadian Health Policy
https://www.canadianhealthpolicy.com/products/evidence-that-regulating-pharmaceutical-prices-negatively-affects-r-d-and-access-to-new-medicines-.html

PMPRB Research Fact Checker

Innovative Medicines Canada
http://innovativemedicines.ca/pmprb-myths/

The Patented Medicine Prices Review Board is Selling Canadians a Lemon: Nigel Rawson for Inside Policy

Nigel Rawson, Macdonald-Laurier Institute
https://www.macdonaldlaurier.ca/is-the-patented-medicine-prices-review-board-selling-canadians-a-lemon-nigel-rawson-for-inside-policy/

Trump signs order pushing to allow drugs to be imported from Canada

Tami Luhby, CTV News, 2020-07-26
https://www.ctvnews.ca/mobile/health/trump-signs-order-pushing-to-allow-drugs-to-be-imported-from-canada-1.5039482

Drug companies warn they will abandon Canadian market if prices reduced

Ryan Tumilty, National Post, 2020-10-15
https://nationalpost.com/news/politics/drug-companies-warn-they-will-abandon-canadian-market-if-prices-reduced?fbclid=IwAR1xSkhcIIeSd0j5kl-9O0d-7LqWSPAzfdL7r6hbNrZjVmV_S0sAjEmrZwE

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