

## Response to Consultation on Proposed Amendments to Patented Medicines Regulations

Submitted by Canadian Organization for Rare Disorders  
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### Rationale for Amendments

The Canadian Organization for Rare Disorders (CORD) is a national registered charity serving as the umbrella organization for rare disease organizations, groups, and patients in Canada. CORD provides a strong common voice to advocate for health policy and a healthcare system that works for those with rare disorders. CORD works with governments, researchers, clinicians and industry to promote research, diagnosis, treatment and services for all rare disorders in Canada.

CORD is strongly supportive of the goal to ensure that Canadians are not paying “excessive” drug prices, interpreted as not “higher than prices paid in comparable jurisdictions” and not “higher than appropriate to assure Canadians have access to medicines optimal to their needs.” That being said, we do not believe that the proposed amendments to the Patented Medicines Regulations provide the appropriate tools and methods to meet the objectives of equitable pricing and patient access. We expressed similar concerns in response to the previous consultation by the Patented Medicines Prices Review Board (PMPRB). We responded at that time to emphasize our concerns with

- Singular focus on drug prices;
- Targeting of “high-cost” therapies which includes many of the drugs for rare diseases and other small patient populations;
- Lack of appropriateness of a “values-based” assessment employing traditional health economics tools and methodologies, especially for innovative or first-in-treatment therapies;
- Overlap with the activities already carried by the Canadian Agency for Drugs and Technologies in Health (CADTH) and Institut national d’excellence en santé et en services sociaux (INESSS)
- Lack of consideration of alternative to budget impact options for assuring cost-effectiveness, including managed access plans and optimal drug utilization (which may be outside of the PMPRB but not Health Canada).

After decades of the PMPRB assuring Canadians that we were NOT being charged excessive prices (based on PRMPB country comparisons), it is rather startling to learn that they were wrong and indeed Canada has “been wronged” perhaps for years. The PMPRB has not put forth evidence based on new evidence or re-analyzed data. What we can deduce, based on the limited documentation provided,

is that Health Canada now believes Canada may be paying “excessive” prescription drug prices based on the following:

- Increasing percentage of healthcare budget to drugs, from 6% (1984) to 14% (today); they note this may be attributed mostly to higher priced drugs and higher utilization but do not note that the increase occurred before 2004 – prescription drugs account for the same share of total health spending today as in 2004;
- Drugs are the second highest category of health spending, more than physician services although the relative shares have been constant over the past decade;
- Third highest average drug prices among 35 OECD countries (only USA and Mexico higher) and the OECD median is 22% lower, but a recent PMPRB report shows that Canadian prices for new active substances (the more innovative drugs) introduced in the last five years ranked in fifth place, tied with Italy, among the seven PMPRB countries, lower than the USA, UK, Germany and Switzerland.
- Increased number (and percentage of spending) of higher priced (>\$10K) drugs, from 42 (2011) to 80 (2016) and from 13.5% to 27.6% (selected drug plans)

Health Canada has proposed amendments that would:

- Use economics-based regulatory factors, i.e., cost-effectiveness analysis including QALY's (quality-adjusted life years) and ICERs (incremental cost-effectiveness ratio), as well cost-utility analysis (CUA)
- Use size of market (potential utilization without restriction)
- Change basket of comparison countries to 12, which would include dropping the USA and adding Australia, Belgium, Netherlands, Norway, South Korea, and Spain
- Get to actual net price by reporting on all indirect price benefits including PLA rebates, discounts, refunds, free goods, free services, gifts or any other benefit in Canada.

Health Canada has also provided a list of questions, which CORD will choose to address only indirectly.

### **CORD Response**

In the opinion of CORD, it is ‘nigh near impossible’ to make a cogent and responsible response to this consultation. There is almost a total absence of health policy and pharmaceutical policy context for the proposed amendments to Patented Medicines Regulations. Before CORD can address whether these amendments are necessary and sufficient to get to “non-excessive” drug prices, we need to know how the proposed amendments will affect Canada’s overall pharmaceutical policy with

regard to access and appropriate utilization. Indeed, it is critical to understand the Health Ministry's policy for pharmaceutical use within the context of the Canadian government's policy for healthcare.

All OECD countries have experienced increases in drug expenditures, notably about 50% increase, and all have taken steps to understand and address the challenges of balancing the benefits of medicines, including new medicines, essential to patient, including those with unmet needs, the impact on overall healthcare costs (both direct and indirect savings as well as expenditures) and the societal value.

It is also exceedingly difficult for us as patient organizations to respond to the consultation given the lack of time and resources at our disposal to address such an important issue meaningfully. Moreover, the Government of Canada has provided us with no background documentation, outcomes of research and analyses, and access to experts or resource persons within the government. We noted at the time of a face-to-face meeting that a single two-hour "question-and-answer" period with a couple of representatives from Health Policy and PMPRB was insufficient to provide us with the context and information necessary to understand the rationale for the proposed amendments and, more importantly, their potential impact on access to therapies.

As patient organizations, we have neither the time nor resources to conduct our own background research, analyses, and consultations. The Canadian Organization for Rare Disorders hosted a consultation inviting policy makers (federal and provincial) as well as payers (public and private), policy advisors, clinicians, patient representatives, and industry. We were grievously disappointed that no one from Health Canada or PMPRB or CADTH accepted our invitation to participate, but we did have representation from all other stakeholders. We will draw upon the learning and recommendations emanating from the consultation.

CORD also took part in a session hosted by the Public Policy Forum where Health Canada and PMPRB were indeed present. We did learn that there was no likelihood of the proposed Orphan Drug Regulatory Framework being implemented, which means that the Government of Canada, unlike the governments of all other developed countries, would not recognize the need for specific legislation to equalize access to drugs for patients with rare diseases. We feel that this omission along with the special attention to the "price" of orphan drugs will serve to further reduce the incentives for researchers and manufacturers to bring drugs for rare diseases and other small patient populations in a timely fashion.

As part of the context for our response, CORD has drawn the following from a relevant current resource on Drug Utilization and Health Policy authored by representatives of institutes in four countries, three of whom are references for Canada (UK, Sweden, Korea, and Denmark). "Health care policy has been defined by the World Bank as the conscious attempt by public officials or executives entrusted

with public funds, including those working in health authorities, health insurance agencies and managed care organizations, to achieve agreed objectives through a set of laws, rules, procedures and incentives.<sup>1</sup> On behalf of the patients with rare conditions, we ask, “What is Canada’s ‘agreed objective’ that the laws and policies are trying to achieve?” We posit there should be only objective, and is the best possible health outcomes for all persons.

We draw from the same source these points with which we agree. “Pharmaceutical policy is a subset of this [health policy], designed to improve the safe and effective use of medicines. Pharmaceutical policy debates incorporate a number of areas, including (i) issues of unmet need, (ii) access to medicines, (iii) pricing and cost containment, (iv) rational use of medicines and (v) innovation and service provision.”<sup>2</sup> We concur that no publicly accountable health authority can consider pricing (affordability) without simultaneously considering access and appropriate utilization. While we acknowledge that the mandate of the PMPRB is limited to control of excessive pricing, this is precisely why Health Canada and the Government of Canada, working with the provinces and territories, must ensure that pricing and affordability are considered within the scope of any proposed changes to laws, regulations, policies, and guidelines that affect cost and availability of patented medicines.

To this end, we note the following key points that are highly relevant here.<sup>3</sup>

- The costs of pharmaceuticals can be controlled in a number of ways, including formal pricing and reimbursement systems, profit controls, patient co-payments, devolution of budgets to physician groups, generic substitution and appropriate use.
- Health care organizations, including governments, can potentially instigate multiple measures to enhance the rational use of medicines. These can be collated under the 4Es: education, engineering, economics and enforcement.
- Rational guidance within pharmaceutical policymaking can only be achieved when policymakers and researchers come together and learn from one another or when initiatives exert their influence in other countries (cross-country learnnag).

### **Comment #1**

It is not surprising that drug expenditures are increasing, primarily because pharmaceuticals are (increasingly) the most effective means of treating disease,

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<sup>1</sup> Godman B, Kwon HY, Bennie M & Almarsdóttir AB. (2016). Drug utilization and health policy. In: Drug Utilization Research: Methods and Applications, First Edition. Elseviers M et al. (Eds.), John Wiley & Sons, Ltd. (pp 203-09)

<sup>2</sup> *ibid.*

<sup>3</sup> *ibid.*

both acute and chronic. Even preventive and surgical interventions rely on pharmaceutical support, whether in the form of vaccination, infectious disease (antibiotic/antimicrobial) control, chronic disease management, pain control, addiction therapy, transplantation, or cellular therapies.

To allow patients and the public to comment knowledgeable on the issue of “not excessive” pricing or “affordability” of medicines, it is imperative that the Government of Canada makes available the analyses as to the impact of the proposed regulatory amendments on drug expenditure and Canadian access to medicines. Will the Government provide other options and scenarios that were considered, those within its direct purview and those outside its jurisdiction (for example, provincial, private, hospital or physician-based)? We would presume that proposed changes have been considered within the context of the potential actions and reactions of other stakeholders.

What are the savings anticipated from the proposed amendments, and can the Government of Canada demonstrate that these actions will not have “unintended” adverse effects, including the timely introduction of innovative medicines? For example, what has the Government concluded as to the likely impact of the focus on pricing (to manage affordability) on access (willingness of companies to engage in clinical trials, to support compassionate access, and to provide timely introduction of new therapies)?

## **Comment #2**

CORD believe that health technology assessments (HTA) that address value are integral to promoting timely and even early access to innovative therapies, especially those for unmet needs where there may be some degree of uncertainty as to real-world health outcomes and safety. We participate with the Canadian Agency for Drugs and Technologies in Health (CADTH), Institut national d'excellence en santé et en services sociaux (INESSS), and individual provincial drug programs to help improve assessment methodology and to provide patient input on individual drugs or technologies. Given that companies already provide comparative and cost-effectiveness analyses (CEA) as well as budget impact analyses (BIA) to CADTH and INESSS and through them to the panCanadian Pharmaceutical Alliance (pCPA), we are unsure what and when these value-based assessments would be delivered to PMPRB. We presume these will be the same analyses only provided earlier (at the time of regulatory submission). We note that many companies already file for “early CADTH” review, even prior to the Notice of Compliance (NOC), so there may be little time delay to market access, so early submission of health economic assessments may not be difficult.

However, this begs the question of who and how the PRMPB will review the CEA and BIA. Presumably, taxpayers will not be footing the bill for another set of assessors. If the review is conducted by CADTH and INESSS, then how will this be

much different than the current process? Currently, manufacturers conduct their CEA based on a “global” price, which may or may not correspond to the PMPRB ceiling price. CADTH/INESSS may request a lower price to meet its CEA (ICER) threshold, which sets the expectations for negotiations with pCPA. The final price, we presume, is based less on PMPRB than on the CEA/BIA analyses (plus size of patient population) and willingness to pay. How will this process change if PMPRB sets an initial “list” price that also includes the ICER?

There may, of course, be some differential impact to the private drug plans, which could claim a lower price upon NOC than the current PMPRB price ceiling. However, this is unclear since the negotiated prices among the private drug plans are also confidential.

We raise the concern that there could be a negative impact on timely access to clinical trials as well as introduction of new medicines if the companies perceive the PMPRB listed price to negatively impact its “global” list price. Based on our previous experience, companies may choose to wait until they have introduced and negotiated prices elsewhere before coming to Canada, if they fear the Canadian price will serve to depress the global pricing. The main losers in this scenario are the patients, including those with rare disorders with no current therapy who are waiting for access to new medicines.

We request to see the analyses that would assure no adverse impact on timely patient access to new medicines and no duplication of work in providing the cost-effectiveness analyses to PMPRB.

### **Comment #3**

We are adamantly opposed to the proposal to introduce the QALY as a standard measure of value and the establishment of an ICER threshold to gauge cost utility and acceptability of new medicines. These concepts were introduced and routinely used when most new medicines were “me-too” drugs for existing therapies. The calculation of the QALY is fraught with controversy, due to many factors relevant to current innovative therapies. For example, for rare diseases with no previous treatment, the calculation of “additional life years” is impossible given that in many cases children typically die in infancy or early childhood and there is no natural history or documentation of disease progression. Similarly, drugs for those near “end-of-life” (e.g., late-stage cancers such as lung or pancreatic which are typically diagnosed at Stage 4) may offer very few “additional months” but these drugs are experienced as extraordinarily meaningful to the patients and families. There is no valid ICER when an innovative therapy is the first treatment for the condition or the innovation replaces an old generic therapy.

Even CADTH and INESSS do not stand by the QALY or ICER when making recommendations on listing. Many of the countries included as the current or

proposed reference list of countries also do not rely on the QALY or ICER, such as Germany, France, Belgium, and Japan. Even the UK, which ostensibly pioneered the QALY and ICER and the reliance on a standards QOL scale (EQ5D) does not strictly abide by these measures, especially for rare diseases or end-of-life therapies. If our reference countries do not manage drug prices with these indices, why does PMPRB need to introduce them here? Why is Canada going backwards?

#### **Comment #4**

CORD welcomes the opportunity to assure that Canadians do not pay “excessive” prices for prescription drugs. However, we cannot comment on the appropriateness of the proposed “basket” of reference countries without receiving analyses from the Government of Canada on the impact of this list, versus potential other baskets. Was this list tested using a sample of current therapies to determine how the Canadian list price and the Canadian (publicly) negotiated price would be impacted by switching to another reference basket? Is the median price relevant and meaningful? Supposedly the list reflects countries with similar GDPs and cost control measures but does the list reflect countries with the same drugs being made available at the same time? We believe not, given that some of these countries reimburse fewer drugs than does Canada, and a few actually fund more. We would expect that the Government of Canada has done that level of research and can provide us with the analyses. We should not be benchmarking average price based on dissimilar drugs with different listing delays following regulatory approval.

Another challenge is that no other country that is referenced here has an entity that is analogous to the PMPRB. Indeed, the prices available for comparison in other countries may be either the manufacturer’s list price or a publicly available negotiated price. Are we really comparing “apples to apples” when we compare Canadian list prices with the prices paid in other countries? We ask the Government of Canada to provide their analyses to assure that we are using comparable indices.

Moreover, we would suggest emulating the drug pricing practices (rather than just aiming for their average prices) of countries with more “ideal” prices and good patient access, such as France or Germany. We ask the Government to provide us with documentation of their analyses and sensitive testing of this list of reference countries.

#### **Comment #5**

CORD emphasizes the need for a clear (national) pharmaceutical policy that is embedded with a national healthcare policy in order for us to discuss cogently the proposed amendments or alternatives to achieving optimal drug prices. It is relatively easy to implement a program that will drive down average drug prices, for example, by not listing innovative therapies or any therapies that are priced

above a specific amount. That is what most developing (low-income) countries do, especially when bringing in universal health coverage and an essential medicines list. But Canada is not a developing country.

One can also set a “fixed” drug budget and only include new therapies when older ones can be supplanted. That is what New Zealand does. The result is that New Zealand patients have access to less than 50% of the new medicines that patients in Australia have. Their relative costs for hospital care exceed those of Australia. Obviously, Canada does not want to be New Zealand.

Some middle-income countries wait until the drugs have been available for many years in more developed countries. They may be able to negotiate access at marginal costs, or even benefit from the introduction of the generic (or biosimilar) version. We have observed this approach in Poland, Croatia, Thailand, and some Latin American countries. Again, these are not Canada.

In Australia, appropriate drug utilization is a key characteristic of their national drug policy. We quote the following:

- The National Medicines Policy (NMP) aims to optimize the use of medicines in order to improve health outcomes for all Australians. It proposes to meet medication and related service needs through four central objectives:
  - Timely access to medicines that Australians need at a cost individuals and the community can afford.
  - Provision of medicines meeting appropriate standards of quality, safety and efficacy.
  - Quality use of medicines.
  - Maintenance of a responsible and viable medicines industry.

Canada does not aim to be Australia but we could and should adopt more enlightened approaches to foster appropriate drug utilization and reporting of outcomes.

#### **Comment #6**

CORD is pleased that the Government of Canada has chosen to consult stakeholders prior to the pre-publication in Canada Gazette, Part 1. We have received information that it is the intention of the Government of Canada to “summarize the results of the current consultations and also include a cost-benefit analysis (CBA) of the proposed amendments that estimates the impact of each element of the proposal on patented medicine expenditures in Canada.” However, we have not been given assurances that there will be a CBA of the potential impact of each element on access to medicines by the target patient populations and optimal utilization.

We request that a CBA of impact on patient access and utilization be conducted prior to the pre-publication of the proposed amendments. More importantly, we request that patient organizations be engaged in the work of conducting the CBA, especially in identifying the relevant patient-centred outcomes and safety concerns as well as measures of utilization (including monitoring, reporting, and re-assessment).

### **Comment #7**

CORD requests that the Government of Canada provide a summary and their analysis of the consultation feedback prior to the publication in Canada Gazette, Part 1. We feel this step is essential; (it would acknowledge the considerable time, effort, and due diligence expended by the stakeholders, promote transparency of the process, and provide assurance that publication does reflect the stakeholder opinions and concerns). It would be even more effective if the Government were to host an all-stakeholders' consultation to share perspectives prior to the publication. The process of multiple stakeholder engagement prior to the Modernization of the Canada Health Act went a long way to assuring that the proposed changes to the Act were fully understood as well as informed by all those affected. It went a long way to establishing trust and cooperation in the implementation. It was a good process and no less valuable now.

### **Conclusion**

CORD welcomes the opportunity for on-going dialogue and consultation with the Government of Canada, Health Canada, and the PMPRB. We do not feel that a focus on controlling the risk of excessive drug prices can be approached separate from assurance of patient access to optimal medicines and appropriate utilization. We also urge earlier, closer, and continuous engagement with the patient and clinical communities to assure all of this work is done with full understanding of the impact on patients and families, for their perspectives, as well engagement of patients to participate in exploring the issues, generate alternative solutions, and implementing consensual approaches that will meet the needs of affordability, access, and appropriate use. We know these optimal pathways exist and we can learn not only from other jurisdictions but each other. We look forward to further consultation prior to pre-publication of the proposed amendments.

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