November 6, 2020

Mr Ron McKinnon, MP
House of Commons
The Standing Committee on Health
Chair
131 Queen Street, Sixth Floor,
Ottawa ON K1A 0A6

Re: Brief regarding the study of the Patented Medicines Prices Review Board's Final Guidelines

Mr Chair,
Committee Members,

I am writing in response to the House of Commons Standing Committee on Health's announcement that it will undertake a study of the Patented Medicines Prices Review Board's Final Guidelines. I would like to express my support for the briefs submitted by Health Charities Coali-tion of Canada (HCCC), the Canadian Organisation for Rare Disorders (CORD), Muscular Dys-trophy Canada (MDC), and the submission signed by MICYRN.

I am also writing as a Canadian citizen living with Spinal Muscular Atrophy (SMA), a rare and debilitating condition that is life threatening in its most severe form. Until 2017 there was no treatment available for SMA. Today, in Canada, there is a first treatment that has been approved by Health Canada and recommended for reimbursement, albeit with differing provisions by prov-ince. There are two more treatments that are currently under review by Health Canada. These are life-saving therapies that have the potential to change the lives of all Canadians affected by SMA. Already, young children are surviving and thriving well past the life expectancy in the nat-ural course of the disease. This future, that brings so much hope to Canadian families, is now threatened by the PMPRB reform. None of these therapies would be accessible to SMA patients in Canada if the Guidelines were already in effect.

The Final Guidelines issued on October 23, 2020 not only fail to address the questions that a majority of stakeholders in the rare disease space have raised during the consultation process, but they also reverse course on an important point of concern for rare disease patients. The decision of the PMPRB to cut the Pharmacoeconomic Value Threshold for Category IV drugs from $150,000/quality-adjusted life-year (QALY) to $100,000/QALY will affect most innovative medicines for rare disorders. This is all the more egregious considering that the federal government has promised a 1B dollars investment towards the development of a Canadian Rare Disease Strategy, a promise that was reaffirmed in the September 23rd Throne Speech.

I urge the Committee to read the detailed letter I submitted to the PMPRB on August 4, 2020, as well as seriously consider the recommendations put forth by the aforementioned organisations.

Sincerely,

Catherine Boivin

Catherine Boivin
Cure SMA Canada Patient Advisory Board

Response to PMPRB Draft Guidelines Consultation
Tuesday, August 4, 2020

Dr. Mitchell Levine
Chairperson
Patented Medicines Prices Review Board
333 Laurier Avenue West, Suite 1400 Ottawa, Ontario K1P 1C1
Email: PMPRB.Consultations.CEPMB@pmprb-cepmb.gc.ca

Dr. Levine
Board Members

I am writing in support of the responses from the Canadian Organisation for Rare Disorders (CORD), Muscular Dystrophy Canada (MDC), and the submission signed by MICYRN and a group of pediatric experts from across Canada. I am not a pharmacoeconomics expert, nor a healthcare practitioner, nor a clinical researcher, but I am a very concerned citizen and patient living with Spinal Muscular Atrophy (SMA). Six months ago, I had no idea the PMPRB even existed and part of me wishes I had never had to learn of its existence. Because if I had not heard of the PMPRB, it would mean that rare disease organizations would not have had to launch an awareness campaign called #Fightforourlives. In the middle of a pandemic, I would not have to also worry about Canadian patients not getting access to new treatments. I would not have to write this letter to beg my government to hear our pleas. Instead, I received an email invitation to attend the spring webinar series hosted by CORD on the regulation changes of the PMPRB. And I was thrust into the complexities of drug approval and pricing regulations in Canada. I was made painfully aware that yet another hurdle was being erected between Canadian patients and access to innovative therapies.

Though I cannot add more expertise to what you have read in the aforementioned submissions, I can ask questions. I can ask you for an explanation. I can let my words look you in the eyes and hope you see the fears of rare disease patients in Canada. This is our last chance to try to make you understand, understand that while you are aiming for fairer drug pricing in Canada, we are fighting for our lives. While you are looking at numbers, charts and data, we are seeing a future where it will be more difficult to get treatment in Canada.

It is startling to even think that we would feel the need to fight for our lives in Canada, nor should we have to. So my first question to you is why? Why leave rare disease patients in this position? **Why not work in a truly collaborative effort with stakeholders in clinical research, patient advocacy and healthcare policy experts towards a framework that supports affordability while improving access to new medications for all?** It is clear from the testimony of people who were invited by the PMPRB at the beginning of the process that you did not allow for alternative approaches or analyses to be suggested either by the Steering Committee or the Techni-

cal Working Group.[1] You welcomed comments but would not engage in a two-way constructive dialog that addressed the many red flags raised by various stakeholders, and in doing so you failed to consider the ramifications and real-world consequences for rare disease patients.

In the June 2020 Backgrounder and in your subsequent webinars, you admitted the need to adjust the previous guidelines to answer to those in the rare disease community and many others who expressed concern about the amount of drugs that would be impacted by the proposed regulations. You adjusted the thresholds that determine Category I medicines and now think that rare disease treatments do not require special consideration.[2] But you only adjusted the thresholds when the submissions to the first round of consultation made you realise you did not have the administrative capacity to deal with the "significant number"[3] of drugs that would be flagged with such low thresholds. So your main consideration for changing your thresholds was not the number of Canadian lives that would be affected but the weight of your workload? How can we trust that you did your homework this time around? Forgive me if I worry that the proper study and consideration might not have gone into the changes you have made. **Your approach seems to be "we'll try it and see what happens,"[4] but do you realise you are playing with lives? We cannot wait to see what happens.** While you might be willing to correct course in the aftermath of implementation,[5] patients are the ones with most to lose. How many chances to procure life-changing therapies will be wasted while you work out the real-world consequences of the revised guidelines?

In light of the first round of public consultations, when many groups and organizations across Canada expressed concerns about the fate of rare disease patients and access to innovative medicines, you thought it wise to address the rare disease community directly with a series of

---

[1] See Dr Tania Stafinski's comments at the Economic Club Canada *Fight for our Lives* conference on her involvement in the Technical Working Group: https://www.youtube.com/watch?v=b5CQYsItUF0&feature=emb_logo @28min55s; also Durhane Wong-Rieger's comments on the Steering Committee in CORD's August 2020 consultation response, and in CORD's June 25, 2020 webinar: https://www.youtube.com/watch?v=amIoWwP6SEI&feature=youtu.be @10min19s

[2] As seen on slide 15 of the PMPRB's July 8, 2020 Public Webinar https://www.canada.ca/content/dam/pmprb-cepmb/documents/consultations/draft-guidelines/2020/PMPRB-Public-Webinar-July8-2020.pdf

[3] Quote from the Backgrounder 2020: "… the observation that the proposed Category 1 criteria would capture a significant number of new medicines is not without merit. As a result, the Board has concluded that in order for its risk-based approach to be **administratively feasible for PMPRB Staff** and patentees, higher thresholds are warranted, both in terms of treatment cost and market size. https://www.canada.ca/en/patented-medicine-prices-review/services/consultations/draft-guidelines.html

4 See comments by Dr Sandy Sehdev, Medical Oncologist at the Ottawa Hospital Cancer Center, in CORD's July 16, 2020 webinar on an admission by Douglas Clark, PMPRB Excutive Director: https://www.youtube.com/watch?v=5qT7ZJXjRNY @36min13s

[5] Quote from the Backgrounder 2020: "Adjustments to the Guidelines will be made if it becomes clear that certain aspects of the new regime are not operating as intended, subject as always to further consultation with stakeholders and the public." https://www.canada.ca/en/patented-medicine-prices-review/services/consultations/draft-guidelines.html

information sessions.[6] You might have thought this was sufficient outreach, yet you provided no opportunities for discussion on alternative approaches. Would it not have been wiser to engage with stakeholders in a truly collaborative way, to take into account their legitimate concerns and listen to their expert recommendations? Why not set up a dialogue to explore different perspectives, to arrive at a mutually beneficial solution? **What would be the harm in saying: we recognize we are not rare disease specialists and welcome your input to ensure our regulations do not bring undue harm to these patients?**[7] Instead you chose to present to us your analysis of data collected around the market and research landscape of rare disease drug development; an analysis that is not only one-sided and misleading, but so obscure[8] as to make it almost impossible for a lay person to understand. Patients are left to our own devices to try to understand how our health will be affected by your unwillingness to consider alternative expert opinions.

You claim that rare disease drugs (DRDs) are increasingly dominating the new drug landscape. **But do you realise that increasing numbers of DRDs reaching late stage development phases means that the years of investment in research are finally paying off? This is what we have been hoping for our entire lives.** That science finally catches up to the disease and that we are able to treat people who previously had no options. Of course there are more specialised therapies reaching approval stages than 10 years ago, and thankfully so! You say DRDs have a higher likelihood of being approved at all stages of development in proportion to other drugs. Do you understand this could be because they answer unmet needs? Because the stakes are higher and because there are much fewer candidates? You say "most DRDs offer limited or unclear therapeutic benefits and are not cost-effective at their list price."[9] But you forget that it is often difficult, sometimes impossible, to set up clinical trials with a large enough cohort seeing as the patient populations are so small and spread out across large geographical regions. It is unfeasible to accumulate the same amount of data as you would with conditions that touch a large proportion of the population. Not to mention that many rare disorders, typically appearing in childhood, are often fatal or require early treatment before the progression is too severe. How can you expect to have longer term studies proving efficacy over time in order to determine cost-effectiveness if patients do not survive in the natural course of the disease? You also fail to take into consideration the qualitative input from patients and caregivers who view

---

[6] Research Webinars given by the PMPRB: https://www.canada.ca/en/patented-medicine-prices-review/services/consultations/draft-guidelines.html

[7] See recommendation in MICYRN's August 2020 consultation response for an Expert Pediatric Advisory Board at the Health Portfolio level; see also comment by Mike Drummond PhD, Professor of Health Economics at University of York, UK, in CORD's July 30, 2020 webinar on seperate specialised committee used by NICE in UK for rare disease specific new drugs: https://www.youtube.com/watch?v=RgQfSu-xRno @21min05s; also comments by Dr Aneal Kahn, Department of Medical Genetics University of Calgary, in same webinar @24min50s

[8] See comments from Neil Palmer, PDCI Market Access, during CORD's June 25, 2020 webinar on how the revised guidelines are even more opaque and complex than the 2019 draft, citing in particular the application of the economic factors and the absence of the formula used by the PMPRB for PE evaluation: https://www.youtube.com/watch?v=amIoWwP6SEI&feature=youtu.be @21min58s

[9] As seen on slide 14 of the PMPRB's June 23, 2020 PMPRB Research Webinar: https://www.canada.ca/content/dam/pmprb-cepmb/documents/consultations/draft-guidelines/2020/Research-Webinar1-EDRD-Market-Size-EN.pdf

these medicines as life changing or life extending.[10] For that matter, you must consider these therapies could provide long term benefits to the whole healthcare system: fewer hospitalisations, less health complications, reduced burden on caregivers, more people able to work, less related mental health problems...[11]

Also, is it really fair to use the list price of a drug in your analysis when we know the actual cost is always negotiated down? **You seem to be depicting a future where DRDs will be the culprit of an overwhelmed healthcare system on the brink of collapse. But you omit putting the spending in context with other health expenditures our system does not question.** According to a report published by Patient Access Solutions on the current and future budget impact of DRDs in Canada, public expenditure on DRDs in 2019, around 280M, was significantly lower than other public health costs like smoking related illness at 6.5B dollars.[12] In light of these discrepancies in methodology, why won't you consider putting in place a multi-stakeholder round-table to arrive at a consensus on the methods that should be employed in the realistic projection of future DRD costs[13] as well as on the ways of evaluating their cost-effectiveness?

In June 2020, following the publication of the new revised guidelines, the Federal Court ruled against the PMPRB being allowed to monitor and report on negotiated prices including the confidential rebates.[14] What is the point then of even setting rebated ceilings if in fact the PMPRB will not be allowed to access the information post-negotiations. If there remains confidential negotiations between manufacturers and public payers after the PMPRB's involvement, then when would the MRPs even come into play? With so much lingering uncertainty, how then can the pharmaceutical companies be expected to know how to plan deployment in Canada?[15] You say developers will still have sufficient incentive to come. You present hypothetical cases showing how the incremental reductions you apply to drug prices reaching above your market thresholds

---

[10] See comments by Lindy Forte, Patient Access Solutions, in CORD's July 16, 2020 webinar on what the PMPRB omitted to include in their 'Insight into the spending on expensive drugs for rare diseases' presentation: https://www.youtube.com/watch?v=5qT7ZJXjRNY @19min41s

[11] See comments by Rosalie Wyonch, Policy Analyst C. D. Howe Institut, on health system opportunity cost in the CORD July 30, 2020 webinar: https://www.youtube.com/watch?v=RgQfSu-xRno @1hr02min05s

[12] See comments by Lindy Forte, Patient Access Solutions, in CORD's July 16, 2020 webinar on the analysis of current and future budget impact of DRDs in Canada, presented at the 2019 International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Meeting : https://www.youtube.com/watch?v=5qT7ZJXjRNY @31min31s

[13] See slide 20 of CORD's July 16, 2020 webinar: https://www.slideshare.net/raredisorders/july-16th-webinar-2-what-we-heard-and-what-we-didnt-hear-at-the-pmprb-public-forum and comments from the webinar by Lindy Forte: https://www.youtube.com/watch?v=5qT7ZJXjRNY @54min30s

[14] See comments on court ruling in CORD's July 30, 2020 webinar: https://www.youtube.com/watch?v=RgQfSu-xRno @9min40s

[15] See comments from Ed Dybka, President and GM for Ipsen Biopharmaceuticals Canada, on industry struggling to understand the new guidelines in the CORD July 30, 2020 webinar: https://www.youtube.com/watch?v=RgQfSu-xRno @1hr04min05s

would still create enough revenue for the company.[16] But you have not provided any reliable, externally validated case-studies demonstrating how your models stand up to real-world examples.[17] **Have you taken every precaution to ensure companies will not be dissuaded from entering the Canadian market early, that patients will not suffer even longer delays to treatment access?** Because we are not dealing in hypotheticals. Rare disease patients deal in survival, in relief from pain, in a race against the clock before irreversible damage and loss. And I have heard both from physicians and researchers that I have personally reached out to as well as from experts on public discussion forums[18] that some companies are already delaying entry into Canada because of the uncertainty and unpredictability surrounding the PMPRB regulation changes. Could they really all be wrong?

You say Canada needs to be more in line with international norms and practices and yet you are pushing regulations further than any other OECD country. Fair and affordable drug pricing is a global concern, and while most countries are looking for ways to address the issue, none are seeking to impose strict price ceilings unilaterally. In fact most OECD countries have flexibility built into their pricing strategies to address specific cases like rare disorders,[19] either through supplemental processes to the standard health technology assessment (HTA) or with modifiers included in the standard HTA in the context of rare disease treatment. So why stick to this method when no one else is using it, especially without properly studying the impacts on patients? **If your objective is to be in line with international median prices, why not start by implementing the changes to the PMPRB 11 comparator countries to set list prices**, which by all accounts seem like it would reduce Canadian public prices by at least 20% and bring us in fact to the OECD median? Further reductions of rebated prices could bring us up to 50% lower than the international median.[20] So why rush into legislation that is in fact outside international

---

[16] As seen on slide 26 of the PMPRB's July 8, 2020 Public Webinar https://www.canada.ca/content/dam/pmprb-cepmb/documents/consultations/draft-guidelines/2020/PMPRB-Public-Webinar-July8-2020.pdf

[17] Quote from CORD's August 2020 consultation response: "Among the most alarming concerns in the updated draft guidelines is the provision for PMPRB staff to arbitrarily modify price tests, resulting in high uncertainty around the application of the economic factors. They have offered no reliable or externally validated examples or case studies to demonstrate how the new system would work. Therefore, despite some changes in the revised draft guidelines, the high uncertainty and threat of lower ceiling prices continue to affect companies' decisions to launch new medicines and invest in health research in Canada." p. 4.

[18] See comments from Jason Field, PDCI Life Science Ontario, during CORD's June 25, 2020 webinar on a study on Canadian vs global launch trends and recent divergence in the Canadian market: https://www.youtube.com/watch?v=amIoWwP6SEI&feature=youtu.be @45min00s; also see MDC's August 2020 consultation response citing two studies, one by Life Sciences Ontario and one by Yanick Labrie (2020) in *Canadian Health Policy*, "Is there any evidence that regulating pharmaceutical prices negatively affects R&D or access to new medicines?A systematic literature review." These studies show a correlation between clinical trial participation and new drug submission/approval; also see CORD's August 2020 consultation response p. 3-4; see also comments by Dr Sandy Sehdev, Medical Oncologist at the Ottawa Hospital Cancer Center, in CORD's July 16, 2020 webinar on cancer drugs not coming to Canada: https://www.youtube.com/watch?v=5qT7ZJXjRNY @37min33s

[19] See comments by Mike Drummond PhD, Professor of Health Economics at University of York, UK, in CORD's July 30, 2020 webinar on how other OECD countries handle pricing strategies for rare disorders: https://www.youtube.com/watch?v=RgQfSu-xRno @17min50s

[20] See slide 6 from CORD's July 30, 2020 webinar: https://www.slideshare.net/raredisorders/july-30-webinar-3-sharing-and-comparing-feedback-for-the-pmprb-guidelines

norms? You say you are following international best practices in the use of HTA methods at a regulatory level, but you neglect to acknowledge that these methods are never used to enforce maximum price caps as requirements for sale, they are used to inform the price negotiation process. You fail to recognise that there is flexibility built into these international systems you hold up as examples to be followed, a flexibility that is lacking in your proposal. **And a lack of flexibility means lives will be lost.**

Which brings me to my final line of questioning. The PMPRB is a regulatory body that monitors prices charged by patentees[21] and may investigate in the case of an egregious filing, but you are not policy makers. **You are not responsible for envisioning and building national healthcare and pharmacare strategies**. You are right that Canada is an outlier among developed countries "with a universal public healthcare system that does not include universal coverage of prescription drugs".[22] Which begs the question, where are the policy experts? Where are the politicians whom are accountable to their constituents? If the federal government wants to build a universal pharmacare strategy that benefits all Canadians and supports the founding principles of our healthcare system of equitable access to care for all, including to those who have been dealt a poor hand in the genetic lottery, then is the PMPRB really the right agency to lead the effort?[23] The federal government even announced in 2019 a 1B dollars investment in a Canadian Rare Disease Strategy to be developed in collaboration with provincial health ministries, clinicians, researchers, policy experts and patient organisations. This important undertaking is meant to encourage research and investments in rare disorders treatment development, as well as better access to and more consistent coverage of innovative therapies.[24] Is the PMPRB not undercutting this effort payed for by taxpayers?[25] **Can we not finish the rare disease strategy before putting up barriers that could deter industry from further partnering with our government in finding affordable solutions for a population of patients in dire need?**

I am one of the 3 million Canadians living with a rare disease, in my case a neuromuscular condition called Spinal Muscular Atrophy (SMA). In 2017, the first ever treatment available for this condition, Spinraza, was approved by Health Canada. Then ensued a long and difficult battle to get accessibility to this expensive therapy to patients across Canada. After much advocacy from the community who saw other countries all over the world getting access before Canada, and

---

[21] http://www.pmprb-cepmb.gc.ca/en/regulating-prices/regulatory-process

[22] Slide 6 from the PMPRB July 8,2020 Public Webinar: https://www.canada.ca/content/dam/pmprb-cepmb/documents/consultations/draft-guidelines/2020/PMPRB-Public-Webinar-July8-2020.pdf

[23] See comments from Fred Horne, Policy Analyst, former Alberta Health Minister, in the CORD July 30, 2020 webinar: https://www.youtube.com/watch?v=RgQfSu-xRno @45min00s

[24] http://www.raredisorders.ca/content/uploads/budget-2019-en2.pdf2.pdf

[25] See comments from Durhane Wong-Rieger on the national strategy for drugs for rare diseases in the CORD July 30, 2020 webinar: https://www.youtube.com/watch?v=RgQfSu-xRno @7min30s; see also comments in MICYRN's August 2020 consultation response on the negative impact the PMPRB reform could have on the significant effort led by the Canadian pediatric research community to streamline timely and efficient clinical trials in Canada and leverage our robust research infrastructure in order to increase the number of clinical trials (p. 3).

after a lengthy negotiation process with all levels of government, CADTH and INESS finally approved Spinraza for reimbursement albeit with varying conditions in different provinces. I am lucky enough to be living in Quebec where the drug was made available to all SMA patients, including adults. I don't want to think what would have happened had the new PMPRB regulations already been in place. Spinraza would most likely not be available in Canada today. I am 8 months into the treatment and already it has not only stabilized the progression of my condition but has reversed the progression by a few years. Every day tasks like eating or brushing my teeth were getting more and more difficult in recent years, and now they are getting easier and easier, not to mention my ability to work longer hours and lead a more productive and fulfilling life. But even without this new therapy I was one of the lucky ones; I made it to 40 years of life. My heart breaks thinking of all the children and families who would have been denied a life-saving treatment for more severe cases of SMA, who up until now did not survive past early childhood.

Because you see, even if you think raising the PE Value and market size thresholds is enough to safeguard drugs for rare disorders,[26] Spinraza would be considered above the 12M annual sales bar you have set. Spinraza has a list price of approximately $375,000/year and must be administered for life. There is an estimated 700 people living with SMA in Canada. Let's assume not all would be eligible to receive Spinraza and cut that number in half. That would still amount to approximate annual sales of 131M. And this is the measure the PMPRB would use to apply strict reductions regardless of the fact that the current paid price is most likely much lower thanks to negotiated confidential rebates. Who's to say the company would have even considered launching in Canada if the new regulations were already in place? I might not be able to remain on Spinraza depending on whether the Quebec government decides to continue funding the treatment for adults based on efficacy outcomes after one year of treatment. In the current system, rare disease patients already have to fight for timely access.[27] **But at least we are given a fighting chance. I have the chance to tell my story, to have a voice in my own care. What will happen to my voice with the new PMPRB regulations?** The current HTA process that informs pricing negotiations with provincial health ministries (INESS and CADTH) allows patient input in assessing the value of a drug, and in some cases in outcome measures used to determine cost-effectiveness in the context of early access through clinical trials or provisional funding. Why not favor an approach like Germany where innovative therapies for small populations are funded on a trial basis and patient input is considered when deciding on public reimbursement strategies?[28] Why would any patient want to continue taking a drug with potential side effects, or a therapy that requires complex medical procedures, if there is no benefit? Our voices matter. In the new PMPRB proposal, where is the space for patient voices? And for input from caregivers and clinicians who understand the qualitative impact of rare disease? **By co-opting**

---

[26] As seen on slide 15 of the PMPRB's July 8, 2020 Public Webinar https://www.canada.ca/content/dam/pmprb-cepmb/documents/consultations/draft-guidelines/2020/PMPRB-Public-Webinar-July8-2020.pdf

[27] See MDC's August 2020 consultation response on the current difficulties for Duchenne Muscular Dystrophy patients to get access to treatments already available in the US and EU.

[28] See Dr Tania Stafinski's comments on Germany's system at the Economic Club Canada *Fight for our Lives* conference : https://www.youtube.com/watch?v=b5CQYsItUF0&feature=emb_logo @1hr00min15s

the prevailing process, the PMPRB is positioning itself as the sole arbiter to determine the value of patient lives.

The bottom line is this: how can we accept to live in a Canada that rips away hope from families who live for the day a breakthrough drug will save the life of their loved one? This is the question I put to you today. Will this government be the one to tell parents that they cannot give their child a life-saving treatment that parents in the US or in Europe have? That they have to watch their child waste away while their friends with the same condition in other countries not only live but are able to walk and run? I expect better from my country. Can we not strive to be leaders? Or at the very least examine and adapt processes actually used in comparable countries that do not jeopardise patients' health? **Can Canada not be the shining light that guides rare disease patients out of the dark? That shows the world how fair access to new therapies is possible through transparent collaboration with stakeholders, accountable private-public partnerships, innovative financing strategies, and a holistic approach to healthcare?**

I thank you for the opportunity to share my thoughts and urge the PMPRB to consider the recommendations put forth by CORD, MDC and MICYRN in their consultation submissions.

Sincerely,

Catherine Boivin

Catherine Boivin
Cure SMA Canada Patient Advisory Board