Patented Medicines Pricing Review Board

Draft Guidelines Consultation

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Submitted By:

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PULMONARY HYPERTENSION ASSOCIATION OF CANADA

L'ASSOCIATION D'HYPERTENSION PULMONAIRE DU CANADA

A better life for all Canadians affected by pulmonary hypertension

Imagine you've noticed yourself becoming increasingly short of breath just going about your daily business. You used to be able to jog up a flight of stairs or walk up a hill without it affecting you. But now you have to stop to catch your breath, and it can be hard to recover sometimes. It's not just the shortness of breath; you're also so tired. Of course you're tired: you have a tough job and people to take care of. And you're almost 50 now. At first you figure you're just out of shape. Admittedly, you have gained a bit of weight and don't exercise as much as you used to. So you make some changes, go back to the gym, and lose 10 pounds. But exercising is so hard, a lot harder than it used to be. It doesn't seem right, so after nine months of symptoms, you go to your GP (bonus: you have a family doctor).

Your doctor doesn't seem too concerned; there are lots of reasons for shortness of breath. You should try and relax; keep exercising. But they prescribe you a puffer too; maybe it will help. You've never had asthma before, but you take the medicine you've been prescribed and start using a puffer throughout your day. It doesn't really seem to help (maybe it takes awhile to work?), and you're still so tired. You're starting to have a hard time keeping up at work, and sometimes when you work out the tips of your fingers turn blue. After six months of no improvement, you go back to your doctor. They're not sure what's wrong, so they refer you to a respirologist (bonus: you have access to a specialist).

It's a six-month wait to see the respirologist; it's now been 21 months since your symptoms began. You're still using the puffer, even though you're pretty sure it doesn't help. But no one has told you to stop using it, and you're worried if you stop your symptoms will get worse. It's getting harder to get through your day. You don't go out as much as you used to; it's too exhausting. Your family has started to notice you're not yourself. The respirologist shares your concern; almost two years of unexplained shortness of breath isn't normal. They order some tests, including an echocardiogram. The echo suggests pulmonary hypertension (PH): high blood pressure in the arteries and blood vessels of the lungs. They refer you to your local PH expert center (bonus: your respirologist knew where to refer you because they received education on the diagnosis of PH from local medical experts and the national PH association).

It takes several more weeks to get an appointment at the PH centre. You've stopped going to the gym; one time you got chest pains and it scared you from going back. You're also on a break from work, since you've been unable to keep up with your job. Google told you that people with PH only live for three years. It's been two years since your symptoms started. How much time do you have? Your spouse comes to your appointment with you. The PH specialist gives you more tests: pulmonary function tests, an EKG, a six-minute walk, and a right-heart catheterization. The tests are difficult, sometimes invasive, and leave you feeling drained and even more terrified. You and your spouse are brought into a small room with the PH specialist and a nurse. They tell you they've confirmed the diagnosis: idiopathic pulmonary arterial hypertension (PAH). Your pulmonary arteries have become constricted and the blood pressure in your lungs is too high; the increased pressure in your lungs is

putting strain on the right side of your heart. They don't know what caused it, but it's progressive, causes heart failure, and there's no cure. You need to begin treatment immediately (bonus: thanks to decades of private research and development there are 10 medications approved in Canada for the treatment of PAH).

You're started on two oral medications and within a couple of months the initial side effects start to fade and you begin to feel somewhat better. You aren't able to return to work, but at least you can still help out at home. You've been learning about your condition: it's rare and, though treatments are available, life expectancy is an average of only 7-10 years. But it used to be even less, so you have reason to hope. You've met people taking a combination of three drugs that have been living with PAH for over 15 years. You know that some of the therapies are difficult to take, involving medication being pumped into your body 24/7 through a device you have to carry around all the time. But that seems like a better option than a double lung transplant. And you've been reading about research in innovative areas: novel therapeutic pathways, improved combination therapies, gene therapy.

Your disease is progressing. Your doctor increases your medications from two to three, but at least you have the option of taking a brand new oral drug before you have to consider a more invasive therapy. There's never been an oral therapy available in this class of drugs before; the side effects are similar to an infusion therapy, but without the risk of infection and a significantly reduced chance of being hospitalized. So you push through the side effects – chronic pain and gastrointestinal distress – and you keep hoping. You know of lots of other conditions where treatment advances are helping patients live longer, healthier lives – HIV, hepatitis C, cystic fibrosis, diabetes, cancer. You're grateful for the researchers doing studies in pulmonary hypertension and for the companies working to develop better therapies. Your family has fewer resources than it used to, but you make small donations to events raising money for PH research. You look for research studies you might be able to participate in. You've heard that patients who participate in clinical trials get free access to the medications.

You've started worrying about what will happen to new medications for PH in Canada. You know governments are looking for ways to lower drug budgets, which makes sense given how expensive health care is today. You know if you had to pay for your own life-saving medications, you wouldn't be able to afford them. What if the government decides it can't afford to pay for your PH medications in the future? It seems like every week there's a story of a desperate patient asking their government to pay for a new medication. If they can't afford it now, what will happen when there are even more new medications on the market? What will happen when it's your turn to benefit from a new breakthrough treatment?

You understand governments are trying to find ways to make health care more sustainable, so you look into the details and discover that the guidelines used by the *Patented Medicines Pricing Review Board* to keep the price of patented medicines

from being "excessive" are being modernized. The changes are complex and very little information is available to patients about the impact that the changes will have on their lives. On the surface, the goal to protect Canadian consumers from unreasonably high drug prices is fair. And it seems like the PMPRB has made considerations specifically for rare diseases – that must be progress! But as someone who has a life-threatening disease - who relies on treatment advances to survive – you have questions:

- How often will the basket of comparator countries be updated? The inclusion of new comparator countries (and the exclusion of the US as an outlier) seems reasonable. But what happens when the international landscape shifts, as it inevitably does? It seems like the cost savings from this policy shift alone will be significant, so why wasn't it done sooner? What mechanisms are in place to ensure that the PMPRB11 list of countries continually contains the most reasonable comparators for the Canadian context?

- The ability of the PMPRB to set a Maximum *Rebated* Price (prior to payers even negotiating with drug manufacturers) based entirely on economic factors sounds really scary to someone with a rare disease! It's clear the new guidelines give some consideration to the different economics of rare diseases, but given the heavy reliance on a cost-utility assessment that is already skewed against rare diseases, how do we trust that these calculations are "fair"? What is considered affordable to government payers rarely translates into what is equitable for those on the margins of society. It is no different for those with rare diseases, a category that will continue to expand as medicine rapidly evolves. How will these new formulas for calculating "value" and "affordability" stand up when applied to paradigm-shifting innovative therapies? How will a curative therapy with an extraordinary upfront cost – a therapy that saves the health care system an extraordinary amount over the course of a person's lifetime – be treated by formulas explicitly designed to radically bring down drug prices and save governments money?

- What mechanisms are in place to monitor and mitigate the impacts – including any unintended consequences – of the new guidelines? Patients deserve to know that if a new policy like this becomes a barrier to access to treatment, there is enough flexibility built into the system to find solutions. For instance, if a drug like Viagra was introduced in Canada today, it's reasonable to conclude that the Maximum Rebated Price would be much lower due to the adjustments required for market size. However, in addition to being a commonly prescribed drug for erectile dysfunction, it was also a game-changing, first-in-class treatment for PAH. A significant price drop would likely create a disincentive for the manufacturer to bring such a drug to the Canadian market, at minimum lowering the urgency and creating delays in access for patients. Such delays have profound effects on the lives of

people with a progressive, life-threatening condition. We may lose function, quality of life, and – importantly – time. It is not an exaggeration to conclude that delays to accessing life-saving medications can cause premature or unnecessary deaths. And so it is not unreasonable for rare disease patients to demand that policies not be applied over broadly or too rigidly. Scientific advancement and breakthrough technologies have the potential to disrupt the most well-intentioned and thought-out policies. What mitigation strategies are PMPRB prepared to undertake to protect the health of those that don't fit neatly into their predetermined formulas?

- How will PMPRB work to gain the trust of rare disease patients, and their families and care providers? This process of modernizing the PMPRB guidelines has been marked by a lack of transparency, confusion, and uncertainty of the future. Rare disease therapies have been viewed only as a problem, never as a solution to the suffering of millions of Canadians. Patients have been asked to trust the PMPRB, even as they proceed with policy changes untested in similar jurisdictions – such as using a set QALY threshold to regulate all prices for all sales. The PMPRB insists it is following international best practices, but its job is not to set international trends, its job is to provide a counterbalance of consumer protection within a policy environment meant to *encourage* pharmaceutical research and development in Canada. These guidelines are only so good as their ability to strike this balance, and ensure that innovation is not stifled and Canada doesn't become a second-tier market for clinical research or new medicines. It is important that the new guidelines be agile enough to proactively respond to progress, rather than becoming entrenched for a generation or more.

For many Canadians, the efforts of the PMPRB are likely seen as both necessary and reasonable. But regulations that make Canada a less attractive market for pharmaceutical companies have a unique and sometimes disproportionate impact on rare disease patients. The PMPRB has signalled its willingness to give special consideration to rare diseases, but vigilance and flexibility will be required to ensure access to new treatments is not sacrificed in an all-encompassing quest by regulators for "affordability". We all want and need an effective, sustainable health care system, but that can only be achieved if we take care not to leave our most vulnerable behind.