Overview

At the Janssen Pharmaceutical Companies of Johnson & Johnson, we are working to create a world without disease. Transforming lives by finding new and better ways to prevent, intercept, treat and cure disease inspires us. We bring together the best minds and pursue the most promising science. We collaborate with the world for the health of everyone in it. We have been an innovator in the Canadian healthcare industry for over 50 years.

We believe that every Canadian deserves equitable access to the most appropriate medicines for their needs, but many patients with rare diseases are facing barriers to accessing these medically necessary medicines.

Our work

Janssen is committed to innovation in Canadian healthcare. We are guided by Our Credo, where our first responsibility is to the patients, doctors, nurses, mothers and fathers, and all others who use our products and services. Our ultimate goal is to help people live healthy lives.

A recent addition to our team is Actelion, an innovator in pulmonary hypertension (PAH), a rare disease which effects roughly 4000-5000 Canadians. Actelion has four products that treat PAH, which is nearly half of the available treatments on the market. Since the introduction of new and innovative PAH therapies, many patients are living longer and healthier lives. These treatments have meant that now, 50% of patients survive five years or more from diagnosis.

But access to these medicines is limited, often with criteria to meet and delays in approval. Some are not yet reimbursed by public payers. These are crucial medicines for those who need them, and delays to access can be devastating. A clear strategy for drugs for rare diseases would help address these issues, better defining what treatments should be made available to patients and ensuring that there is funding available to pay for them.

Medicines for rare diseases

According to the Canadian Organization for Rare Disorders, there are almost 3 million Canadians with rare diseases, and 60% of them are children.[1] These diseases are often disabling or life-threatening and involve a long list of challenges in accessing the right diagnosis, care and treatments.

In the case of Pulmonary Arterial Hypertension (PAH), it is a serious rare illness that affects the blood vessels that carry blood from the heart to the lungs. Everyday simple tasks that we all likely take for granted can feel as difficult as climbing Mount Everest for PAH patients. Patients have told us that this disease has a significant effect on their quality of life and makes them feel like "second class citizens" – they must depend on others to help them. Based on the results of

[1] https://www.raredisorders.ca/about-cord/

the International PAH Patient and Carer Survey supported by Actelion, it was revealed that the impact on those surveyed, reaches far beyond the physical symptoms of PAH.[2]

A recent survey showed that nearly 80% of rare disease patients experience challenges in accessing necessary medicines.[3] This is a substantial issue that needs to be tackled head on by governments to ensure that all Canadians have access to the best possible medicines and treatments for their needs.

These barriers to access come because of uncoordinated and uneven approaches across the country to both regulatory access and reimbursement of these medicines.

RECOMMENDATIONS:

- The federal government should implement an orphan drug regulatory framework, which would encourage the development and availability of orphan drugs in Canada.
- The federal government should fund a separate drug program, specifically for drugs for rare diseases.

Federal initiatives

Janssen is extremely concerned that two ongoing initiatives of the federal government will further accentuate the issue of access to drugs for rare diseases. These initiatives, the proposed reforms to the Patented Medicines Prices Review Board (PMPRB) and the implementation of National Pharmacare, while well intentioned, could unintentionally restrict access to new innovative medicines.

RECOMMENDATION:

- Closely consider the implications of the proposed revised Patented Medicines Pricing Board regulations and National Pharmacare to ensure that these do not have unintended negative consequences, including hindering access to drugs for rare diseases.

Patented Medicines Prices Review Board reforms

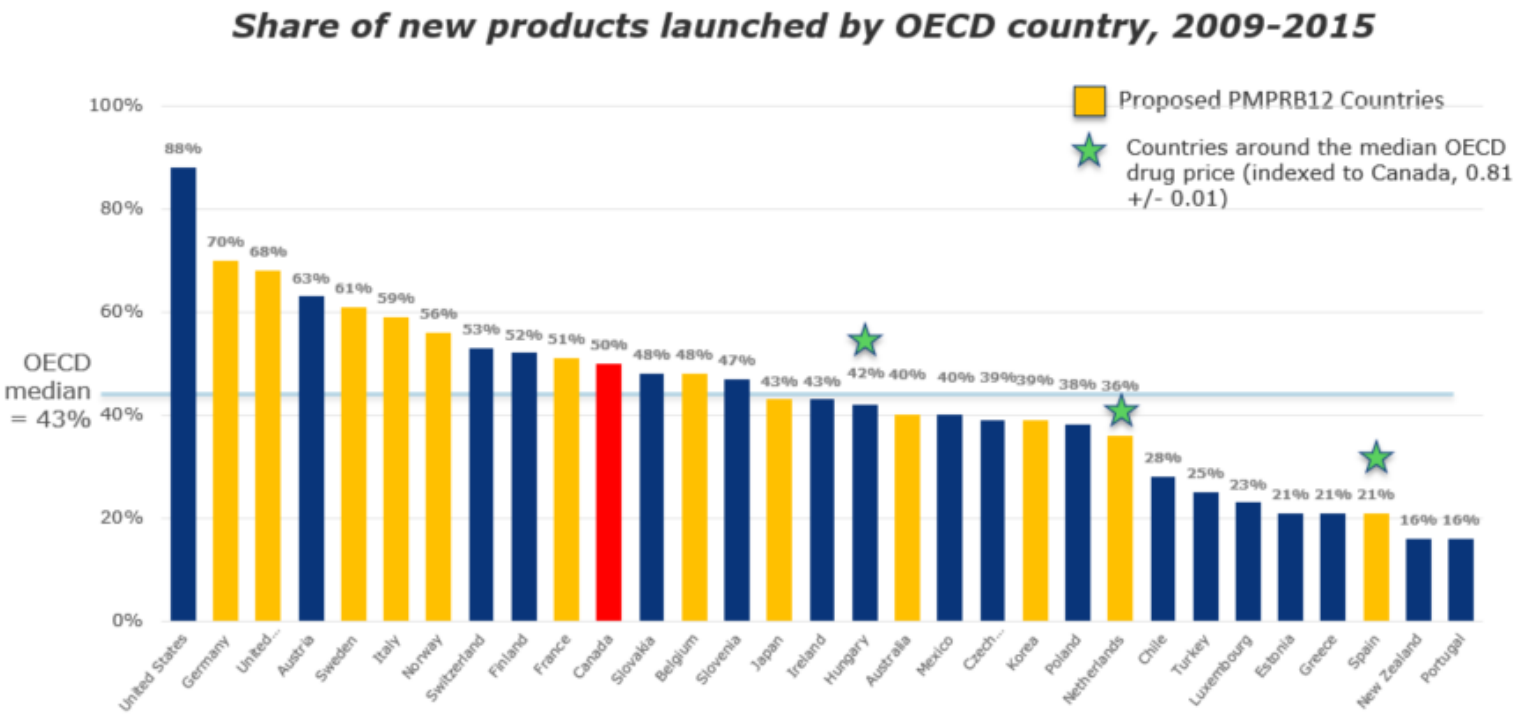
The proposed PMPRB regulations, as they currently exist, will decrease access to new innovative drugs, especially drugs for rare diseases.

These reforms, which would drastically lower the prices of patented drugs in Canada, threaten access to new medicines by lengthening the time it will take for pharmaceutical companies to introduce these medicines into this market, and some may not launch at all. Health Canada and

[2] http://www.phaeurope.org/wp-content/uploads/PAH_Survey_FINAL.pdf
[3] Canadian Organization for Rare Disorders. Pharmacare Survey 2018.

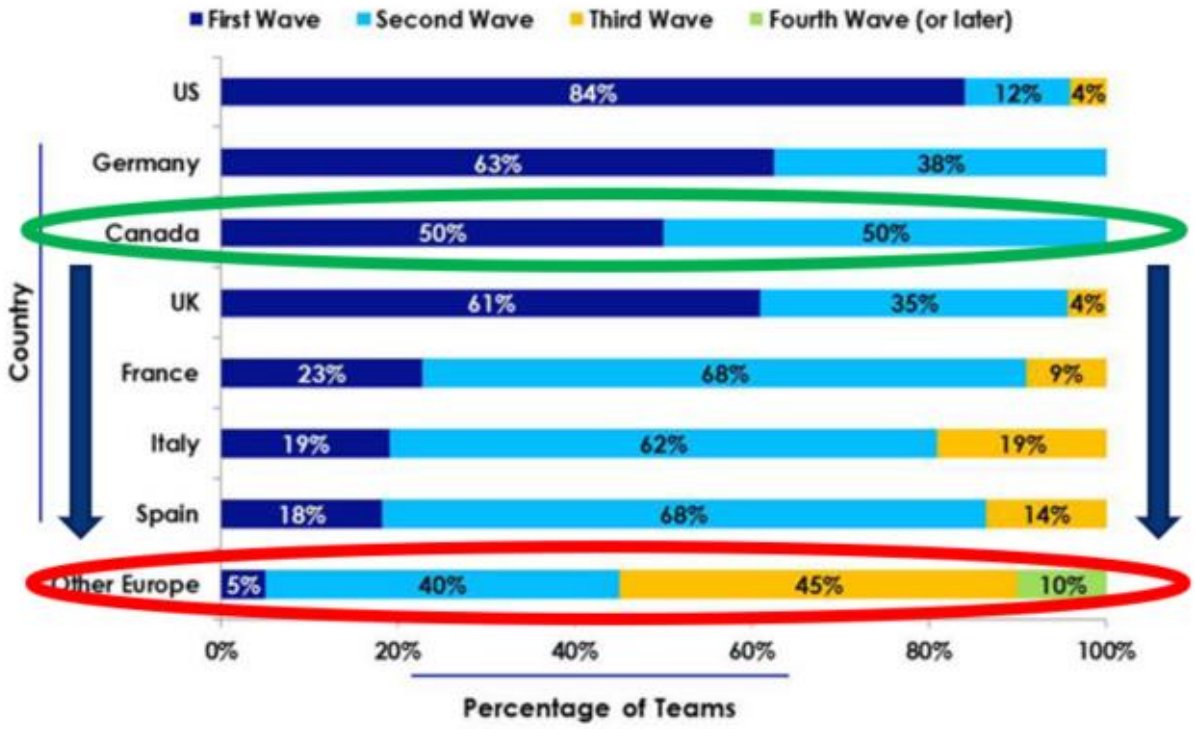
the PMPRB have indicated that the target price for medicines should be the OECD median. If this is the case, Canadians will have to accept levels of access to innovative medicines similar to that of countries with pricing that reflects the OECD median, which means that there will be less access to new medicines than Canada.

The proposed changes will result in less new innovative medicines launching in Canada altogether. It has been seen that less medicines are launched in countries with prices at the median of the new proposed comparator countries.



[4] PMPRB Meds Entry watch 2016 report. http://www.pmprb-cepmb.gc.ca/view.asp?ccid=1374&lang=en; countries around the OECD median added by Janssen.

Additionally, the updated regulations will stop Canada from being an early launch country, slowing access to necessary innovative medicines that launch in Canada.



[5]

Some will argue that many medicines that are not available in the new comparator countries are not innovative and therefore not important to patients. While not all medicines are important to all patients, patients respond differently to different medicines, even those in the same class. Therefore, it is important to ensure the right medicine gets to the right patient at the right time. This is particularly important for drugs for rare diseases.

It is important to note that any delay for patients living with life threatening illnesses may make the difference between a patient surviving or not surviving.

National Pharmacare

Janssen believes that patients and physicians should have the best possible access to medications and be able to choose the drugs that provide the best treatment for the individual patient based on her/his particular needs. Patients with rare diseases have unique needs and require medicines that are often not listed by public drug plans.

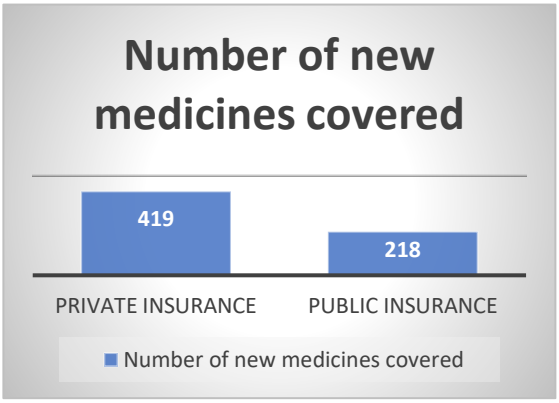
Maintaining patient and physician choice of medicines should be an underlying value that supports the design of any new Pharmacare policy or program. For this reason, it is important

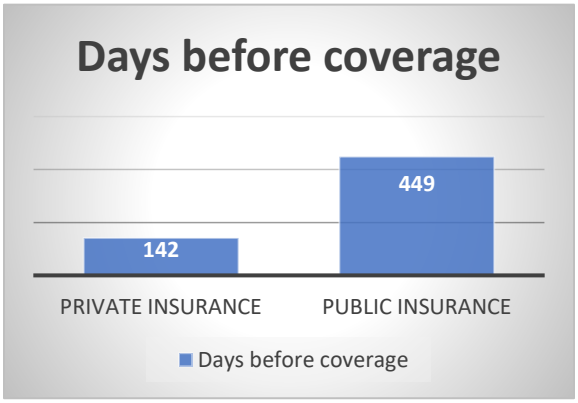
[5] http://www.marketwired.com/press-release/us-germany-and-uk-among-most-common-first-wave-markets-in-launch-sequencing-2194318.htm

that current private drug insurance plans stay in place because they traditionally provide more choice than public plans and are much quicker to cover new innovative therapies.

To do this, Canada should maintain the existing public/private mix of drug plans to ensure that no one loses access to the drugs they currently have access to. Private coverage also will ensure faster coverage of new innovative medicines as they come onto the market.

A 2018 Canadian Health Policy Institute report showed that more medicines were covered by private insurance plans as they came on to the market. It noted that "of the 479 new drugs approved by Health Canada from 2008 to 2017, 87% (419) were covered by at least one private drug plan compared to 46% (218) that were covered by at least one public plan, as of June 30th, 2018."[6]





The same Canadian Health Policy Institute report also showed that new medicines are listed much quicker by private insurance. It noted that "averaged across all years studied, private drug plans took 142 days to cover new drugs compared to 449 days for public drug plans."[7]

The ability of patients and physicians to choose the most effective medicine to treat an individual patient must continue if we want to sustain and build upon the strengths of our current healthcare system. A single-payer Pharmacare system would impose a much smaller formulary for reimbursement, based on current experience with public formularies. This would dramatically reduce drug access for Canadian's with rare diseases.

[6] "Coverage of New Medicines in Public versus Private Drug Plans in Canada 2008-2017." *Canadian Health Policy,* August 20, 2018. Canadian Health Policy Institute.
[7] Ibid.

Moving forward

Thank you to the House of Commons Standing Committee on Health for undertaking this incredibly important study into rare diseases and for allowing Janssen to contribute to this most valuable work. The results of these deliberations can have a positive impact on nearly 3 million Canadians and can set an excellent example of how policy should be implemented to ensure that all patients have the ability to access innovative treatments on a timely basis in Canada.

Improving access for medicines for rare diseases should be a key pillar of any rare disease framework and should not be impeded by other federal initiatives such as the Patented Medicines Prices Review Board reforms or the implementation of National Pharmacare. Both of those initiatives should be looked at through a critical lens to determine if they are truly going to improve access to medically necessary medicines, or if they will severely impede access to medicines Canadians currently have access to, as well as, to future innovative medicines.