Submission to the Federal Standing Committee on Health regarding the PMPRB guideline changes

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The Politics of Patient Advocacy Groups and the PMPRB Regulations and Guidelines

Prepared by Sharon Batt, co-founder of Breast Cancer Action Québec; author of *Health Advocacy Inc.: How Pharmaceutical Funding Changed the Breast Cancer Movement*

Breast Cancer Action Québec/*Action Cancer du Sein du Québec* is a patient advocacy group whose mandate includes informing women and the general public about scientific research on breast cancer and treatments.[1] BCAQ/ACSQ was founded by four breast cancer patients in 1991. Today, the organisation continues to speak for patients and the wider public, in both official languages.

We welcome this opportunity to address the House of Commons Standing Committee on Health. Our brief on the new guidelines of the PMPRB discusses the politics of industry funding. Many patient organisations today receive support from the pharmaceutical industry, to the extent that these relationships can seem normal. We don't think they are. Financial partnerships with the pharmaceutical industry challenge a group's ability to advocate for policies that serve the public interest. Since 2001, Breast Cancer Action Quebec has had a written policy explaining why we don't accept funds from the pharmaceutical industry and certain other corporations.[1]

Main Points

- Pharmaceutical industry funding of patient advocacy groups is a growing concern in international health policy circles.
- Industry-funded patient groups say the PMPRB changes will discourage companies from carrying out clinical trials in Canada and deny patients access to new drug therapies. This claim confuses clinical care with clinical research. It masks the fact that a new treatment may have no benefit and may cause significant harm.
- Industry-funded patient groups express fears that companies will not bring new drugs to Canada if the PMPRB changes go into effect. Vertex Pharmaceuticals has fanned these fears by refusing to register its new cystic fibrosis drug in Canada. Patient groups in other countries have confronted Vertex and brought the company to the negotiating table. Health systems globally are at risk because of unethical drug prices; governments, public health agencies and civil society need to act in concert to effect systemic change.
- Research in the United States shows that "patient assistance programs" are an industry strategy to maintain high prices and these programs are integrated with industry-funded patient organisations. To ensure that the PMPRB changes and national Pharmacare work as intended, greater transparency is needed to clarify how these programs work in Canada and their effect on drug prices.
- Partnerships between pharmaceutical companies and patient organisations contribute in myriad ways to inflate drug prices and to skew patients' advocacy in the industry's favour. Patient groups are political actors and need to find other sources of funding.

Industry Funding of Patient Advocacy Groups: A Global Concern

Pharmaceutical industry funding of patient organisations is a major policy issue internationally. The pharmaceutical industry now funds patient groups in high-income countries, including the US, the UK, Australia, Sweden, Ireland, throughout Europe, as well as Canada.

In the United States, researchers found that upwards of 80 per cent of patient organisations receive some money from the pharmaceutical industry.[2] in 2015, 14 companies collectively spent US $163 million on patient advocacy groups. This was two-and-a-half times the $63 million that the same companies spent lobbying politicians the same year.[3] Industry-funded patient groups sponsored a newspaper ad campaign that opposed legislation to contain prices of drugs covered by U.S. Medicare. As with the PMPRB, the groups claimed they would be denied access to "cutting edge" therapies if the changes went through.[4] In Missouri, six opioid manufacturers paid almost $9 million to patient groups between 2012 and 2017. The groups echoed and amplified industry messages favourable to opioid use.[5]

Members of patient groups in Australia described their industry relationships as an exchange of mutually beneficial assets. The companies gave the groups money, information, and advice. The groups provided the companies with opportunities to build relationships with patient opinion leaders. They coordinated drug access lobbies with companies. They enhanced company credibility and helped companies with clinical trial recruitment.[6] In Canada, the U.K, and Australia, patient advocacy groups participate in the national agencies that recommend whether or not a new treatment should be publicly funded. The potential value of their opinions is undermined because most receive funds from the same companies whose drugs are under review.[7]

WILL THE PMPRB CHANGES DENY PATIENTS ACCESS TO DRUGS IN CLINICAL TRIALS?

Form letters from patient advocacy groups to MPs and the PMPRB describe clinical trials as an opportunity for patients to benefit from innovative therapies.[8] These groups and the industry argue that changing the PMPRB regulations and guidelines will deny innovative therapies to patients because companies will cease conducting clinical trials in Canada. This argument is profoundly unethical and contrary to Canada's Tri-Council policy governing human research. The mistaken belief that new drugs will benefit patients is called the therapeutic misconception.[9] It confuses clinical care (what's best for the patient) with clinical research (assessing the value of a treatment). This false belief masks the fact that a new treatment may have no clinical benefit and may cause significant harm.

The essential question for patients is not whether trials are run in Canada, but whether they are run ethically. Good clinical trials advance our understanding of the best treatments and benefit future patients. But unethical clinical trials that create distorted knowledge are well-documented.[10] They include ghost-written studies, poor informed consent, trials that are too short or too long, and trials with negative results that are not reported. These trials harm patients.

Patient advocacy groups can educate their members to avoid poor trials and support good ones. They can work with researchers to encourage ethical trials in Canada. They can refuse to participate in trials unless the sponsor agrees to post the trial results, even if the trial shows the treatment was ineffective or harmful. They can monitor trials and publicize those that have trial results posted, or not. They can refuse to participate in trials unless sponsors commit to pricing new therapies affordably, according to the PMPRB guidelines.

WILL COMPANIES REFUSE TO BRING DRUGS TO CANADA BECAUSE OF THE PMPRB CHANGES?

Patient advocacy groups express fear that the PMPRB changes will discourage companies from bringing new treatments to Canada. This fear is underlined by a harsh current reality: the American company Vertex has refused to register the new cystic fibrosis treatment Trikafta in Canada, denying the treatment to CF patients here. This is an outrage, because Trikafta could benefit 90% of Canada's 4,300 cystic fibrosis patients. Vertex claims the PMPRB changes are the reason it is withholding Trikafta from Canadian patients. The company says it will not make enough profit to continue its research under the new PMPRB rules. Overwhelming evidence suggests this is not true. Independent patient groups in other countries have successfully challenged Vertex's pricing practices.

Trikafta is the fourth "modulator" drug for cystic fibrosis that Vertex has brought to market since 2012. The drugs improve function in the mutated gene that causes cystic fibrosis. A research team led by Dr. Lap-Chee Tsui at the Hospital for Sick Children and the University of Toronto discovered the CF gene in the 1980s. The Canadian Cystic Fibrosis Foundation and CF clinics in Canada identified almost all the research subjects, from families in Canada. The families donated blood samples for this ground-breaking research. The Canadian Cystic Fibrosis Foundation, the Sellers Fund of Sick Kids, and the tax-funded National Institutes for Health (NIH) in the US, all supported the research financially.[11]

Vertex: the good, the bad and the ugly

Vertex provides a case example of how patient organisations can exert political influence for the public good – or fail to do so. Vertex had no interest in cystic fibrosis until the American Cystic Fibrosis Foundation (CFF) approached the company in 2000. The CFF wanted a company to translate Dr. Tsui and his colleague's basic research into an effective treatment and eventually a cure. It gave Vertex $150 million for research and development. Like its Canadian counterpart in the 1980s, the CFF raised money from its community and sourced its patient network for research subjects. The result: first, a series of effective treatments, launched under the brand names Kalydeco, Orkambi, Symdeco and Trikafta; second, a financial bonanza for both Vertex and the CFF, which received a share of royalties. In late 2014, the CFF sold its royalty rights to an investment firm for $3.3 billion. Sadly, the Foundation has not leveraged its influence to insist Vertex make its drugs accessible to the global CF community. Critics wonder if its financial conflict of interest explains its silence.[12] The CFF's president, Robert Beall, said that asking Vertex to price Kalydeco more affordably "would have been a dealbreaker."

Vertex's exorbitant prices have shocked the world. The company launched Kalydeco in the US at $294,000 annually for two pills a day. Twenty-nine researchers and physicians who had

worked to develop the drug wrote to Vertex's CEO to express "dismay and disappointment" that the triumph of developing a successful drug was "diminished by the unconscionable price." The company, they said, "could appear to be leveraging pain and suffering into huge financial gain for speculators" including the companies top executives, reported to have made millions in a single day."[13] Aiden Hollis studied Vertex's pricing for Klydeco and Orkambi and estimates the company's profits from the two drugs will be $21.1 billion. He concludes, "the high prices are not justified by costs or the need to support innovation. Instead, the prices seem more designed to reward shareholders."[14]

Some Canadian patients have turned their anger towards the federal and provincial governments. In BC, where the provincial government refused to cover Orkambi on its health plan, a patient launched a class action lawsuit.[15] But is the government at fault, or Vertex? The company's willingness to "leverage pain and suffering" is a problem for governments everywhere, not just Canada. We need health policies that serve the public good – all patients, not just those who lobby aggressively, often with corporate support.

International challenges to Vertex

Some patient groups in other countries have successfully challenged Vertex, rather than their governments. In the UK, inspired by the American film The Dallas Buyers' Club, parents of CF patients formed a buyer's club to collectively purchase bioequivalent CF modulator drugs from Gaydor, a generic company in Argentina. They checked the company's safety standards, which were high. To help families cover the costs of the imported drugs, they partnered with an experienced charity. Sample prices of bioequivalent CF drugs from Gaydor are US $18,353 per patient per year for the Kalydeco equivalent (versus US $300,000 in the U.S.); and US$14,965 for the adult dose of the Orkambi equivalent (versus US $259,000 per patient per year in the U.S.)[16] Gaydor hopes to have a biosimilar version of Trikafta by the end of next year.

The group Just Treatment, also in the UK, launched its "Plan B for Orkambi" campaign, when Plan A (NHS negotiations with Vertex) failed. The group consulted Andrew Hill, a specialist in generic drug prices at the University of Liverpool, to obtain target generic prices for Orkambi for the UK market. They estimated that generic Orkambi could be profitably supplied for £5,000 per year, compared to the UK list price for Orkambi of £104,000 per patient per year. They demanded that the UK government issue a compulsory licence to make generic Orkambi available to UK patients. The compulsory licence threat brought Vertex to the negotiating table and a deal with the NHS was reached. In the Netherlands as well, adverse publicity and the threat of a compulsory licence broke a negotiating deadlock.[17]

In the United States, the Institute for Clinical and Economic Review (ICER), a Boston-based health technology assessment agency, evaluated Trikafta and asked for public comment. Patient groups and patient family members had varied responses. ICER awarded Trikafta an "A" for clinical effectiveness and concluded that no insurer, private or public, should or would deny the drug to American CF patients. Like all other drug assessment agencies, ICER concluded the price was set too high. ICER urged insurers to use its critique to bargain for price reductions. Notably, key points of contention from the public are similar to the competing public responses to the PMPRB changes. More than half the comments objected to the use of QALYs, the statistical method for determining the opportunity costs of an intervention's price on the health

system. Several organisations said that ICER's assessment would "stifle innovation," a comment also voiced in response to the PMPRB changes. One intervener, however, emphasized that "not all Cystic Fibrosis Families condemn cost-benefit analysis" and urged ICER to be wary of critiques of such analyses coming from groups funded by Vertex. Another urged ICER to go further than the use of QALYs in assessing Vertex's pricing of its drugs and "consider the role of the patient community, taxpayers and government in the invention of new drugs."[18]

Our views are in line with the two latter opinions. The role of civil society organisations is to work in the public interest, for the health of all, not to be the squeaky wheel for our own subgroup at the expense of others. We applaud organisations like Just Treatment and VertexSaveUs that have found workarounds to contest Vertex's unfair pricing. If all patient organisations and health advocacy organisations acted in globally solidarity to contest excessive drug prices, these unfair practices would have to change.

Legislative bodies like HESA and health regulatory bodies like the PMPRB, are likewise guardians of the public health. Vertex's first obligation, like all private corporations, is to its shareholders so of course Vertex seeks to maximize its profits. We need you to put in place a better system than the one we have now, a system that protects patients from price exploitation, and from ineffective and unsafe drugs. As the lawyer and ethicist Jonathan Marks has argued, society needs to jettison the idea of public-private partnerships in health. We need to devise a new paradigm, one in which governments, health agencies and civil society health groups go mano-a-mano with private interests, instead of thinking we can work hand-in-hand.[19]

COSTLY DRUGS AND PATIENT ASSISTANCE CHARITIES: WHOSE INTERESTS DO THEY SERVE?

The PMPRB has rightly dropped the United States from its comparator countries. The U.S. health system and its drug prices make the country an outlier among high-income countries. Nonetheless, since Canada does not include drugs in its national public health system, issues of drug access have parallels in Canada and the U.S. Patient assistance charities, which help patients pay for expensive drugs, are a case in point. These organisations are different from the classic community-based patient organisation, but they work in concert with them and are part of the patient support and advocacy landscape.

To my knowledge, these organisations have not been subject to special investigations in Canada. In the United States, however, they have come under scrutiny. Here I sketch why these organisations have attracted American regulators' and journalists' attention. They are drug access strategies that deserve HESA's attention because they help keep prices inflated.

A typical patient assistance program covers insurance co-payments and may help a patient pay insurance premiums. The money comes entirely from pharmaceutical companies or from other charities they fund. Co-pays are intended to encourage patients to switch to lower-priced generics, but this incentive is removed if an outside source covers the co-pay. A study by Citi Research found a pharmaceutical company donation of $1 million to help patients stay on high-cost drugs could generate up to $21 million for the company.

U.S. Federal authorities have investigated the assistance charities under anti-kickback laws and fined one pharmaceutical company $210 million.[20] The Office of the Inspector General of the Department of Health and Human Services has investigated the programs for evidence that they influence which drug patients take. Between 2001 and 2014, pharmaceutical company spending on patient assistance charities increased from $2.3 million to $1.4 billion. In a 2014 Bulletin, the Inspector General's Office said it would increase its scrutiny of arrangements between the pharmaceutical donor and the "independent" charities that provide patient assistance. Because charitable donations reduce the companies' tax bills, they are a subsidy that costs the US treasury billions of dollars in foregone tax revenue, in addition to maintaining high prices that cost public payers like Medicare.[21]

A 2015 Senate Finance Committee hearing on the soaring cost of specialty drugs found that, in setting the price of a new specialty drug, one company wrote off the costs of donations to Patient Assistance Programs as operating expenses, "a cost of doing business." The Office of the Inspector General concluded that the growth of Patient Assistance Programs keeps patients from abandoning expensive new drugs, even when other, equally effective and less costly, alternatives are available. If a company faces criticism over price increases, the programs provide public relations cover. The company Mylan, for example, raised the price of its two-pack Epipen epinephrine autoinjectors from $100 to about $600 and responded to public outrage by increasing its patient assistance programs.[22]

Critics say these collaborations keep drug prices and other health costs high. David Mitchell, who heads Patients for Affordable Drugs, an American patient group independent of the pharmaceutical industry that advocates for lower drug prices, describes the groups as "a marketing arm of pharma." He says the charities mask the price of drugs, which drives up the cost of healthcare. Insurance companies pass the costs on to consumers and employers and patients are "caught in the middle," says Mitchell. Patients who depend on expensive drugs are grateful for the programs, even if they realize they are not ideal. In the words of a patient with multiple myeloma, "someone has to have the guts to stand up to the pharmaceutical companies."[21]

Conclusion and Recommendations

Partnerships between pharmaceutical companies and patient organisations contribute in myriad ways to inflate drug prices and to skew patients' advocacy in the industry's favour. Canada needs a national publicly funded drug plan and policies to support it.

Breast Cancer Action Québec recommends:

- That the new PMPRB guidelines go into effect on January 1, 2021. These new guidelines will help reduce the excessive cost of medicines in Canada and prepare the ground for a national publicly funded universal Pharmacare plan;
- That patient organisations be required to declare the source, amounts and purpose of all funding from the pharmaceutical industry in written submissions to federal health committees and/or agencies;
- That organisations selected to represent patients and the public on panels, committees and other federal bodies established to advise the government on pharmaceutical policy free of funding from the industry.

Conflicts of interest declaration for Sharon Batt

In 2017-2020, Sharon Batt received payments to speak at St. Paul's University (Ottawa), at the Nova Scotia Health Coalition AGM (Halifax), on a Writers' Union of Canada book promotion tour (Montreal and Toronto), and at the University of Windsor. The Jean Monnet Network for Health Law and Policy at Dalhousie University paid her travel expenses to speak at the Mario Negri Institute in Milan and the Canadian Agency for Drugs and Technologies in Health provided a patient advocate grant to the CADTH Annual Symposium in 2018. She receives royalties from UBC Press for sales of a book she wrote. She has had writing contracts with the Canadian Federation of Nurses's Union, PharmedOut (Georgetown University, Washington DC), and a Workshop at Dalhousie University (Halifax). The Canadian Health Coalition supported the writing of this brief.

In addition to being a co-founder and honorary member Breast Cancer Action Quebec, Sharon Batt is a member of the executive board (Secretary) of the Nova Scotia Health Coalition, a member of Independent Voices for Safe and Effective Drugs, a volunteer member of the Public Awareness Committee of the Canadian Deprescribing Network, and a volunteer member of the Scientific Advisory Committee on Health Products for Women, Health Canada.

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