PATENTED MEDICINE PRICES REVIEW BOARD

IN THE MATTER OF the *Patent Act*
R.S.C. 1985, c. P-4, as amended

AND IN THE MATTER OF
Horizon Pharma (the "Respondent")
and the medicine Cysteamine Bitartrate sold by the Respondent under the trade name PROCYSBI

WRITTEN SUBMISSIONS OF THE RESPONDENT, HORIZON PHARMA PLC IN RESPECT OF THE DECISION: *Alexion Pharmaceuticals Inc. v. Canada (Attorney General)*, 2021 FCA 157

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HORIZON'S SUBMISSIONS

1. The Federal Court of Appeal's decision in *Alexion* provides important guidance on three issues affecting this case.

2. First, the Court clarified that the proper approach to determining excessiveness under section 85 of the *Patent Act* begins with construing the Board's statutory mandate. The Board's mandate is not consumer protection or price regulation, as Board Staff has asserted in attempting to discharge its onus. Rather, the Board's mandate is to prevent the abuse of a patentee's patent monopoly.

3. Second, the Court cautioned that any departure from the Guidelines must be consistent with section 85 of the *Act*. The Board cannot depart from the Guidelines to achieve a result that is inconsistent with the text and purpose of section 85, which is aimed at pricing that amounts to an abuse of the patent monopoly.

4. Third, the Court reiterated that the Board must provide explicit reasons to explain and support its analyses. A failure to provide detailed reasons, nourished by a proper understanding of the Board's mandate and founded upon the *Act*, with clear reasons justifying departures, if any, from the Guidelines, will be considered to be unreasonable.

5. Finally, *Alexion* is a decision that is presently binding and must be faithfully interpreted and applied.

A. The Board's mandate is to prevent excessive pricing caused by an abuse of the patent monopoly

6. **Board Staff has misconstrued the Board's mandate in seeking to discharge its onus**. In this proceeding, Board Staff urged upon this Panel that the Board's mandate is consumer protection and nothing more. *Alexion* makes clear that this wrong.

7. In *Alexion*, the Court explained that the Board's statutory mandate is to prevent abusive (*i.e.*, excessive) pricing that may arise by virtue of an abuse of the monopoly power given by a patent.[1] The Board's mandate is not one of consumer protection; the Board cannot engage in general price control or regulation.[2] *Alexion* makes clear that Board Staff's position in this case is premised on a fundamental misapprehension of the Board's mandate. Simply put, Board Staff cannot meet its onus because its case rests on the erroneous premise that the Board's purpose is consumer protection and price control.

8. On the other hand, Horizon urged the Panel to consider the objectives of the *Act*, including the "important balance" between "incentivizing research and development of patented medicines and their introduction into Canada through the grant of a monopoly and protecting against abuse of that monopoly" (a purpose recognized and embraced in *Alexion*[3]). While Board Staff argues that this was "wrong in law,"[4] *Alexion* confirms that it is Board Staff that is wrong. Horizon also claimed that the Panel could not focus exclusively on consumer protection.[5] Board

[1] *Alexion Pharmaceuticals Inc. v. Canada (Attorney General)*, 2021 FCA 157, at paras. 11, 49-51 ["*Alexion*"]

[2] *Alexion*, at paras. 49-51

[3] *Alexion*, paras. 50, 56; Horizon Closing Submissions, paras. 286, 331; Horizon Reply Submissions, paras. 87, 96-97, 133

[4] Board Staff Reply Submissions, para. 9

[5] Horizon Closing Submissions, paras. 286-288; Horizon Reply Submissions, paras. 87-99

Staff said this, too, was wrong. Instead, it maintained: "The Board has one mandate – that of consumer protection."[6] Following the unequivocal language in *Alexion*, Board Staff is wrong about this too.

9. The Court's clarification of the Board's mandate has several practical implications for this case.

10. **Board Staff has failed to meet its onus**. Board Staff has the onus of proving that the price of PROCSYBI is excessive under section 85. This burden is on Board Staff and this burden never shifts. Board Staff, throughout the investigation and the prosecution of this hearing, has pursued a consumer price protection mandate. (As set out in more detail below, Board Staff's witness, Mr. Kellison, acknowledged that Board Staff was focused on setting a "reasonable" price for PROCYSBI).[7] *Alexion* makes it clear that this is misguided. In discharging its onus, Board Staff must establish that Horizon abused its patent monopoly and that the price of PROCYSBI (which it alleges is excessive) was "*made possible by the abuse* of the monopoly power" given by its patent.[8]

11. Board Staff not only failed to tender evidence of patent abuse, but it ignored this element of the Board's mandate altogether. Instead, it erroneously focused on its own view of the "reasonableness" of PROCSYBI's price and consumer protection. This resulted in not one untested pricing methodology (as was the case in *Alexion*), but three proposed pricing methodologies, all of which are inconsistent with and exceed the Board's mandate under the *Act*.

[6] Board Staff Reply Submissions, para. 9

[7] Kellison Transcript, Volume 3, p. 561, ll 13-15, p. 575-576, ll 23-25, 1-4

[8] *Alexion*, para. 11

Board Staff's failure to demonstrate excessiveness in the context of an abuse of the patent monopoly is fatal to its case.

12. **"Excessiveness" must be tied to patent abuse**. The Court confirmed that there must be a nexus between an excessive price and the abuse of a patent monopoly. Indeed, it characterized an excessive price as that which is *"made possible* by the abuse of the monopoly power given by a patent."[9] This nexus is central to the Board's mandate. Following *Alexion*, the Board cannot reasonably discharge its mandate without determining whether an excessive price was "made possible by" the "abuse of the monopoly power given by a patent."[10]

13. To make this determination, the Board must consider whether there is evidence of patent abuse. This requires the Board to engage and consider the evidence of the economic implications of Board Staff's proposed prices in the specific context of this case, which includes the special challenges inherent in developing and pricing rare disease drugs. The Board cannot determine whether the price of PROCYSBI is excessive – in the sense that the price is an abuse of the patent monopoly – without considering how economic and clinical forces inform the pricing of an ultra-rare disease drug. As explained below, this evidence fits squarely within the analysis that the Board is required to undertake pursuant to section 85 of the *Act*.

14. **Price must be considered within the relevant context**. Under section 85(1)(a), the Board must consider the prices at which the patented medicine has been sold. For the Board's analysis to be meaningful, this factor – the price of the patented medicine, PROCYSBI – must be considered within the relevant context. Here, the relevant context includes Horizon's evidence

[9] *Alexion*, para. 11

[10] *Alexion*, paras. 11, 49-50

about the economic and clinical considerations which necessarily inform the pricing of ultra-rare disease drugs, such as PROCYSBI.

15. The Board cannot make a reasoned determination about whether the price of PROCYSBI is an "abuse of monopoly power" without considering this evidence. Indeed, as the Court stated in *Alexion*, drugs to treat ultra-rare conditions often take decades to develop and can be expensive. The mere fact that a drug is "expensive" is not enough: even a "very expensive" price "says nothing about whether the price is 'excessive' within the meaning of section 85."[11]

16. **The outcome must be consistent with the Board's mandate**. Horizon tendered evidence of the economic implications of Board Staff's proposed prices. Horizon tendered this evidence to show that (i) it has not "abuse[d] the monopoly power given by [its] patent,"[12] and (ii) Board Staff's methodologies produce a result that is fundamentally at odds with the Board's mandate.

17. The evidence showed that Horizon will not only fail to recover its costs and earn a return, but it [redacted] at Board Staff's proposed prices.[13]

18. This evidence is consistent with (i) a contemporaneous, third-party analysis undertaken by KPMG, and (ii) the evidence of Board Staff's expert, Mr. Rosen.[14] While Mr. Rosen readily agreed that Horizon [redacted] under Board Staff's Same Medicine Test, he did not calculate Horizon's [redacted] using his "preferred" methodology.[15] When

[11] *Alexion*, para. 54

[12] *Alexion*, para. 11

[13] Horizon Closing Submissions, paras. 171, 178

[14] Horizon Closing Submissions, para. 173

[15] Horizon Closing Submissions, para. 191; Exhibit D20-A, Expert Report of H. Rosen – October 6, 2020, para. 6.22(d)

Horizon performed this calculation (*i.e.*, using Mr. Rosen's preferred methodology), the results showed that Horizon would [redacted] under Board Staff's proposed pricing models.[16] This evidence is central to the Board's mandate as defined in *Alexion*.[17]

19. When the Panel examines the evidence, and properly interprets and fairly applies the legislation to the evidence, there cannot be a reasoned explanation for an outcome that results in Horizon incurring [redacted]. In the words of *Alexion*, this outcome is "irrational on its face" and is "evidence that the outcome cannot be supported by the facts and the law."[18] Put simply, this outcome reflects an approach that was fundamentally tainted by a misconstruction of the Board's mandate. The logic underpinning Board Staff's alternative models lacks probity: each of the proposed tests exceeds the "permissible statutory mandate" by "regulating the reasonableness of pricing" rather than preventing the abuse of a patent monopoly.[19]

B. Any departure from the Guidelines must be consistent with section 85

20. The Court in *Alexion* emphasized that any departure from the Guidelines must be reasonable and consistent with section 85.[20] Given the importance of this decision to the patentee, the Court also reiterated that the obligation on the Board to explain its decision to

[16] Horizon Closing Submissions, para. 241; Rosen Transcript, Volume 14, p. 3299, ll 14-21
[17] *Alexion*, para. 33
[18] *Alexion*, para. 31
[19] *Alexion*, para. 11
[20] *Alexion*, paras. 39, 57

depart from the Guidelines is "high."[21] If the Board departs from the Guidelines, it must have a logical and justifiable basis for doing so.[22]

21. Importantly, the Board must not rely on factors, or "special circumstances," whose only purpose is to achieve a specific result. Yet, this is precisely the type of "results-oriented" analysis in which Board Staff engaged, and which it asks this Panel to accept in this case.[23] Board Staff reverse-engineered three new pricing tests to achieve a specific result – namely, to drive the price of the patented medicine PROCYSBI down to the price of the unpatented medicine Cystagon. None of these approaches is founded upon the Guidelines or is grounded in the *Act.*

22. Board Staff's justification for departing from the Guidelines appears to be that (i) PROCYSBI's level of therapeutic improvement does not fit within the existing categories in the Guidelines; and (ii) even if it did fit within the existing categories, the price test for a "moderate improvement" is not appropriate because the price of PROCYSBI, in Board Staff's view, is too high.[24] As was the case in *Alexion*, neither of these reasons "logically support" departing from the Guidelines. Rather, they underscore the result-oriented nature of Board Staff's methodology.

23. **No logical basis to depart from the Guidelines**. There is no support for the claim that PROCYSBI does not fit within an existing therapeutic category. Indeed, asking the Panel to make this determination would require it to ignore:

[21] *Alexion*, paras. 39, 59
[22] *Alexion*, paras. 12, 37, 39, 62
[23] Horizon Closing Submissions, para. 334
[24] Board Staff Closing Submissions, para. 250

(a) binding jurisprudence – including the Federal Court of Appeal's recent decision in *Galderma* – on the meaning of the "[patented] medicine";[25]

(b) Dr. Dohil's first-hand and hands-on evidence about the fourteen years of research and development that led to the invention of a delayed-release formulation of enterically-coated, microspherized beads of cysteamine bitartrate known as PROCYSBI;[26]

(c) HDAP's recommendation that PROCYSBI is at least a "moderate improvement" (although we note that, throughout, Horizon has maintained that the level of therapeutic improvement is (at least) substantial);[27] and

(d) Dr. Langman's real-world, scientific evidence about PROCYSBI's therapeutic effectiveness;[28]

(e) the medical literature addressed by both clinical experts in this case – Drs. Langman and Midgley – that speak to the significant clinical improvements of PROCYSBI over Cystagon.

24. **Board Staff adopted a result-oriented approach**. There is no logical basis for Board Staff's radical departure from the Guidelines. Board Staff departed from the "moderate improvement" test early in its investigation of PROCYSBI's price because of purportedly "unique circumstances." Those unique circumstances all revolve around the price of

[25] Horizon Closing Submission, paras. 274-279
[26] Horizon Closing Submissions, paras. 21-46
[27] Horizon Closing Submissions, paras. 111, 325
[28] Horizon Closing Submissions, paras. 47-75

PROCYSBI, even though its price in Canada is commensurate with the price in Europe (and is less than its price in the United States).

25. A rush to the lowest international price was expressly rejected in *Alexion*, where the Court held that "[i]t is not enough to allude vaguely to 'unique circumstances' and then just name two circumstances that do not appear to be unique and that fall short of logically supporting the sort of significant, unprecedented departure from the Guidelines the Board took here."[29] Yet, in this case, Board Staff has completely abandoned a consideration of PROCYSBI's international prices and has chosen instead to adopt three unprecedented pricing tests benchmarked to the current price of the unpatented medicine Cystagon.

26. The allegedly "unique circumstance" put forward by Board Staff in this proceeding is the price differential between Cystagon and PROCYSBI.[30] This is not a unique circumstance, nor does it logically support departing from the Guidelines. It simply led to an outcome that Board Staff did not find acceptable, not for any evidentiary basis, but because Board Staff was of the view that the price of PROCYSBI was "unreasonable" in relation to Cystagon. As Mr. Kellison admitted on cross-examination:

> "We started from the premise that the test for moderate improvement in the guidelines might not be appropriate in this case"
>
> […]
>
> "Again, I'd say this is a situation, sort of looking at the guidelines we were working with, where it was the **view of Board Staff that the tests that are laid**

[29] *Alexion*, para. 63

[30] Board Staff Closing Submissions, para. 272

> **out in those guidelines didn't generate what we'd consider to be reasonable results**, given the facts of this particular case."[31]

27. Because the application of the Guidelines did not produce the result it sought, Board Staff proceeded to reverse engineer three new pricing tests that would "generate results" in the "order of magnitude" they were looking for.[32] Each of Board Staff's tests is designed to force the price of PROCYSBI down to the current price of the unpatented Cystagon. Unsurprisingly, Horizon fails to cover its costs and make a return under each of these tests.

28. Board Staff's exclusive focus on these reverse-engineered pricing tests is devoid of the purpose and mandate of the *Act*. Put simply, it is an attempt at price control, not the valid exercise of the Board's authority to prevent the abuse of a patent monopoly through excessive pricing. As a result, Board Staff has failed to demonstrate that the price of PROCYSBI exceeds the maximum non-excessive price permitted. To the contrary, the current price of PROCYSBI – benchmarked to international prices and based on a rigorous application of the Guidelines – is not an abuse of the patent monopoly and is not excessive.

29. Putting aside the fact that the Board lacks jurisdiction to decide what price is "reasonable," accepting Board Staff's result-oriented approach will lead the Panel into error. *Alexion* is clear: departing from the Guidelines to achieve a specific result (in this case, to reduce the price of PROCYSBI to Cystagon's current price) "would be a ground for setting aside its decision."[33]

[31] Kellison Transcript, Volume 3, p. 561, ll 13-15, p. 575-576, ll 23-25, 1-4

[32] Kellison Transcript, Volume 3, p. 561, ll 16-19

[33] *Alexion*, paras. 49, 62

C. The Board must provide explicit reasons

30. Finally, the Court reiterated that in exercising its discretion under the *Act*, the Board must provide explicit reasons grounded in the *Act*.[34] The Board must explain the basis for its determination of excessiveness or non-excessiveness under section 85, having regard to the factors in s. 85 and the evidence brought to bear on those factors.

31. In *obiter*, the Court pointed to the language in sections 85(1) and (2) as an example to show that, before concluding its analysis under section 85(2), the Panel must provide reasons as to why it cannot determine whether a price is excessive under section 85(1). As stated above, when one considers the economic losses that Horizon would suffer, the Panel is well-equipped to conclude why the proposed price tests are unworkable to arrive at a conclusion of excessiveness.

32. The Court also reiterated that when considering costs under section 85(2), the Panel must have evidence. That evidence was lacking in *Alexion*. The only evidence was the inference proffered by Professor Schwindt that, in putting a drug on a market at a price, the patentee must be taken to be covering its costs and earning a return. (In *Alexion*, the Court was critical of this approach as it led to the adoption of a Lower International Price test that "read out" the prices of the other countries that had to be considered.)

33. While the evidence in *Alexion* was lacking, in this case Horizon has tendered that evidence. The Panel must consider this evidence and provide clear reasons explaining why it can or cannot decide the matter under section 85(1) alone.

[34] *Alexion*, paras. 58-59, 63-64

D. *Alexion* must be followed

34. The time within which the Attorney-General may file an application to seek leave to appeal the Alexion decision to the Supreme Court of Canada expires September 29, 2021. Any such leave application, or a decision on a leave application or, assuming leave is granted, decision by the Supreme Court of Canada is speculative at this point in time. At this point in time, Alexion is the law and needs to be faithfully interpreted and rigorously applied.

ALL OF WHICH IS RESPECTFULLY SUBMITTED this 10TH day of September 2021.

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