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

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

Torys LLP
79 Wellington St. W., 30th Floor
Box 270, TD South Tower
Toronto, ON M5K 1N2
Fax: 416.865.7380

Sheila R. Block
Tel: 416.865.7319
sblock@torys.com

Andrew M. Shaughnessy
Tel: 416.865.8171
ashaughnessy@torys.com

Rachael Saab
Tel 416.865.8172
rsaab@torys.com

Stacey Reisman
Tel: 416.865.7537
sreisman@torys.com

Counsel to Respondent,
Horizon Pharma PLC

CONFIDENTIAL-CONFIDENTIEL and s. 87 *Patent Act* Privilege

TO: **The Secretary of the Patented Medicine Prices Review Board**
Standard Life Centre
333 Laurier Avenue West
Suite 1400
Ottawa, ON K1P 1C1

Toll-free: 1.877.861.2350
Direct Line: 613.288.9635
E-mail: Sherri.Wilson@pmprb-cepmb.gc.ca

AND TO: **Perley-Robertson, Hill & McDougall LLP/S.R.L.**
Constitution Square
1400-340 Albert Street
Ottawa, Ontario
K1R 0A5

David Migicovsky
Tel: 613.566.2833
dmigicovsky@perlaw.ca

Christopher P. Morris
Tel: 613.566.2802
cmorris@perlaw.ca

Courtney M. March
Tel: 613.566.2859
cmarch@perlaw.ca

Counsel to Board Staff

AND TO: **MINISTRY OF JUSTICE**
Legal Services Branch
P.O. Box 9280 STN PROV GOVT
1001 Douglas Street
Victoria, BC V8W 9J7
Fax: 250.356.8992

Sharna Kraitberg
Tel: 250.356.8931
Sharna.kraitberg@gov.bc.ca

Lawyer for Her Majesty the Queen in Right of the Province of British Columbia, as represented by the Minister of Health Representative for the Intervenors, the Provinces of Manitoba, Ontario, and Newfoundland and Labrador

HORIZON'S REPLY SUBMISSIONS

1. In its submissions, Board Staff has ostensibly invited the Panel to ignore the Federal Court of Appeal's decision in *Alexion*. This approach will lead the Panel into error.

2. Board Staff's invitation to disregard *Alexion* manifests in several different ways:

   (a) it erroneously asserts that its conception of the Board's mandate was always consistent with the directives in *Alexion*, and at the same time argues that the Board's mandate as described in *Alexion* is inconsistent with what it says is "binding guidance" from the Supreme Court of Canada that the mandate of the Board is consumer protection;[1]

   (b) having tailored its evidence to its misconstrued consumer protection mandate, it now proclaims without any basis that its experts and pricing methodologies were *always* focused on excessive pricing in the sense of patent abuse, and that the Board can rely on these to justify a departure from the Guidelines;[2] and

   (c) it asserts that there is a "bright line" between sections 85(1) and 85(2) of the *Act*, implying that the Board can somehow discharge its mandate to determine whether the price of PROCYSBI is an abuse of the patent monopoly without considering its economic and clinical context.[3]

[1] Board Staff Submissions on *Alexion* dated September 10, 2021 ("Board Staff Alexion Submission"), paras. 20, 24-29

[2] Board Staff Alexion Submissions, paras. 16-18

[3] Board Staff Alexion Submissions, para. 23

3. Board Staff's attempts to both (i) distance its arguments in this case from the same errors it urged upon the Court in *Alexion* (which the Federal Court of Appeal resoundingly rejected); and (ii) argue that *Alexion* is wrong in law are plainly without merit.

4. First, Board Staff misconstrues the Supreme Court of Canada's decision in *Celgene*, which considered whether the Board had jurisdiction over a drug made available through the Special Access Programme. Although *Celgene* and *Alexion* are different types of cases, the decision in *Alexion* is consistent with *Celgene*: both cases affirm that the Board's mandate is to ensure that patentees do not abuse the patent monopoly by charging excessive prices to Canadian consumers.

5. Second, *Celgene* did not address excessiveness under the *Act*. *Alexion* is the leading appellate decision addressing the scope of the Board's mandate in the context of the meaning of "excessiveness" in a pricing case under section 85. Contrary to Board Staff's suggestion, *Alexion* is binding and must be faithfully interpreted and applied.

6. Third, while it implores the Board to disregard *Alexion*, Board Staff suggests that it can somehow recast its evidence in this case as consistent with the mandate identified in *Alexion* and as a basis for departing from the Guidelines. It is not. Unsurprisingly, Board Staff has made the same errors that it urged the Court to adopt in *Alexion*. It fashioned evidence aimed not at showing that the price of PROCYSBI was an abuse of its monopoly, but that the price is "unreasonable" in relation to Cystagon. This evidence is incapable of forming a reasoned basis for departing from the Guidelines.

7. Finally, Board Staff's suggestion that sections 85(1) and (2) are separated by a "bright line" fundamentally disregards the mandate of the Board as described in *Alexion*. As the Court

acknowledged in *Alexion*, the mere fact that a drug is "expensive" does not ground a finding of excessiveness, and the Board cannot make a reasoned determination about whether the price of PROCSYBI is an "abuse of monopoly power" without considering the economic and clinical context of ultra-rare disease drugs.

8. ***Alexion* is consistent with *Celgene*.** As stated in Horizon's submissions dated September 10, the Federal Court of Appeal in *Alexion* made clear that the Board's mandate is to prevent excessive prices caused by patent abuse. Board Staff suggests that the mandate described in *Alexion* is inconsistent with *Celgene*. It is not. In *Celgene*, the Supreme Court affirmed that the Board is responsible for ensuring that the monopoly that accompanies the granting of a patent is not abused.[4] This is consistent with the decision in *Alexion*, in which the Federal Court of Appeal reiterates that the consumer protection purpose referred to in *Celgene* is tied to the specific need to prevent patent abuse.[5]

9. Board Staff's suggestion that there is some inconsistency between the Board's mandate as articulated in *Alexion* – to prevent excessive pricing caused by the abuse of the patent monopoly – and the broader goal of consumer protection identified in *Celgene* defies both logic and common sense. Horizon does not dispute that the provisions of the *Act* which give the Board its jurisdiction over the price of patented medicines protect consumers from excessive prices. Of course they do – the Board is protecting consumers by preventing excessive prices. But this does not mean that the Board may engage in price control measures that are unconstrained from the goal of preventing the abuse of a patent monopoly.

[4] *Celgene Corp. v Canada (Attorney General)*, 2011 SCC 1, headnote and para. 29 ["*Celgene*"]
[5] *Alexion Pharmaceuticals Inc. v. Canada (Attorney General)*, 2021 FCA 157, para. 49 ["*Alexion*"]

10. Board Staff relies on a quote from *Celgene* that highlights this distinction. It says that the Board is entitled to be "guided by the consumer protection goals **of its** mandate" as if it had a singular, consumer protection mandate.[6] Clearly, the Board's mandate is not consumer protection. The Board's mandate – and thus the central issue in this case – is whether the price of PROCYSBI is excessive in the sense that the price is an abuse of the patent monopoly. The fact that this determination is made, in part, to protect consumers does not change the scope of the Board's statutory mandate under the *Act*. Consumer protection does not trump the objective of encouraging innovative medicines into the public domain, which is why the Court of Appeal in *Alexion* held that excessiveness is not tied to the "reasonableness" of the price in the eyes of the consumer, but to the abuse of the monopoly granted by a patent.

11. While Board Staff recognizes that the *Act* provides a balance between incentivizing research and development and the need to protect against the abuse of excessive prices, it argues that it is not the pricing system overseen by the Board but the *Act* "read as a whole" that strikes that balance. In essence, as it argued at the hearing, Board Staff continues to suggest that other provisions of the *Act* address the problem of incentivizing patentees and that section 85 is directed exclusively to consumer protection.

12. This is clearly wrong on a plain reading of *Celgene* and *Alexion*, which both direct the Board to balance the interests of the monopoly granted by a patent with the interests of consumers. In *Celgene*, the Supreme Court upheld the Federal Court of Appeal's decision, which held that the very "purpose of the provisions of the *Patent Act* creating a system for regulating the price of patented medicines is to strike a balance between the public interests in encouraging

[6] Board Staff Alexion Submission, para. 26

research and the development of new medicines through the award and protection of a patent, and 'the need to ensure that Canadians have access to patented medicines which are reasonably priced.'"[7] It is not other parts of the *Act* that achieve this balance: it is this very section of the *Act*. That is why the Federal Court of Appeal in *Celgene* stated that Board's mandate "is to ensure that patentees do not abuse the monopoly created by the grant of a patent with respect to a medicine by charging excessive prices to consumers in Canada."[8] This is fundamentally consistent with the Federal Court of Appeal's decision in *Alexion*.[9]

13. At the hearing, Board Staff pursued a singular consumer protection mandate that focused on its view of the "reasonableness" of PROCYSBI's price in relation to Cystagon. Board Staff is now faced with having to explain away the decision in *Alexion*, which expressly clarifies that the "consumer protection purpose" referred to in *Celgene* is tied to "the specific need to prevent patent abuse", because the decision leaves Board Staff without a rationale for its pricing proposals.[10] Board Staff cannot cherry-pick statements from *Celgene* in an attempt to discard *Alexion* and manipulate the scope of the Board's mandate.

14. ***Alexion* is the leading case interpreting excessiveness under the *Act.*** Board Staff attempts to hang its entire case on the fact that the Supreme Court referred to the broader goal of consumer protection in *Celgene* as evidence that the Court in *Alexion* is somehow wrong about the Board's jurisdiction to determine excessiveness under section 85 of the *Act*. This is wrong – not only because the decisions are consistent on the nature of the Board's mandate (as set out

[7] *Celgene*, para. 29; *Canada (Attorney General) v. Celgene Corporation*, 2009 FCA 378, para. 48 ["*Celgene FCA*"], citing *ICN Pharmaceuticals, Inc. v. Canada (Patented Medicine Prices Review Board)*, 1996 CanLII 4089 (FCA), para. 3 ["*ICN Pharmaceuticals*"]

[8] *Celgene FCA*, para. 16

[9] *Alexion*, para. 49

[10] *Alexion*, para. 49

above) – but also because *Celgene* does not purport to provide an interpretation of the meaning of excessiveness under section 85.

15. *Celgene* must be read in context. The "single issue" in that case was a threshold jurisdictional issue: the meaning of the phrase "sold in any market in Canada" under the *Act* and its impact on the Board's jurisdiction over medicine entering Canada through the Special Access Programme.[11] The Supreme Court did not consider the scope of the Board's statutory mandate under the *Act* when interpreting the meaning of "excessiveness" in a pricing challenge under section 85. The other decision to which Board Staff refers, the Federal Court of Appeal's decision in *ICN Pharmaceuticals*, considers the Board's mandate in the context of whether a patent "pertains" to a medicine and is therefore subject to the Board's jurisdiction.[12] Neither of these cases directly engage the issue of excessive pricing.

16. *Alexion* is the leading appellate decision directly addressing the scope of the Board's mandate in the context of the meaning of "excessiveness" in a pricing dispute under section 85 of the *Act*. Try as it might, Board Staff cannot escape *Alexion*: it is the law and must be faithfully interpreted and rigorously applied.

17. **No reason to depart from the Guidelines.** Board Staff's suggestion at paragraph 17 of its submissions that the Guidelines are "not directed to the tribunal's decision maker" is wrong. As the Court in *Alexion* held, "the Board has enacted [the Guidelines] to assist itself and others in applying section 85."[13] Horizon agrees that the Guidelines are not binding on the Board.

---

[11] *Celgene*, para. 1

[12] See *ICN Pharmaceuticals*, paras. 1, 29

[13] *Alexion*, para. 38

However, as *Alexion* makes clear, any departures from the Guidelines must be reasonable and must be accompanied by a reasoned explanation.[14]

18. Because Board Staff has pinned its entire case on a faulty understanding of the Board's mandate, there is no evidence to ground a reasoned explanation for its departure from the Guidelines. Board Staff suggests that its evidence from the hearing – premised on a mandate of consumer protection and three pricing methodologies that wrongly required PROCSYBI's price to be "reasonable" in relation to Cystagon – is somehow consistent with the Court's directive in *Alexion* and can be repurposed to ground a basis for a departure from the Guidelines. It is not. Professor Schwindt's evidence, in particular, was premised on Board Staff's pricing methodologies and the assumption that the price of PROCYSBI should be "competitive" in relation to Cystagon.[15]

19. Professor Schwindt's evidence is inconsistent with the Court's direction in *Alexion* that excessive pricing is not considered in the context of "competitiveness" or consumer protection, but the abuse of a monopoly granted by a patent. Not only is this evidence not a reasoned basis for departing from the Guidelines, it is incapable of establishing that PROCYSBI's price is excessive as defined in *Alexion*. To the contrary, the evidence demonstrates that PROCYSBI's

---

[14] *Alexion*, para. 39

[15] Expert Report of Professor Schwindt dated September 6, 2019, pp. 14-15. See, for example, Schwindt Transcript, pp. 833-835 where he stated: "Well, with respect to excessive prices, we will call those prices at which a firm will earn monopoly profits or monopoly rate. So essentially, an excess of price is a price that would – that does exist above what would exit in a competitive market." Professor Schwindt went on to say that the "markets then will generate a competitive price which sees to it that all firms are efficient, they're achieving the lowest cost possible, those that haven't, have been removed from the market, and it's a form of stability. When prices get above that, they become, in an economist's perception, as excessive."

See also Schwindt Transcript, p. 854. Professor Schwindt admitted that he is "not qualified to make statements about the pharmacology of these two medicines" (i.e., PROCYSBI and Cystagon) and that the "core issue is the comparability of these two medicines, PROCYSBI and Cystagon, and it's a scientific question which is out of my realm of expertise."

price – set at the median international price of the PMPRB's reference jurisdictions – is non-excessive under section 85(1).

20. **No bright line between sections 85(1) and (2).** Board Staff asserts that the Court in *Alexion* delineated a "bright line" between the consideration of evidence going to sections 85(1) and (2). As Horizon explained in its September 10 submissions, this is an incorrect interpretation of the Court's *obiter* comments because it fundamentally disregards the mandate of the Board as described in *Alexion*. As the Court acknowledged in that case, the mere fact that a drug is "expensive" does not ground a finding of excessiveness: the Board cannot make a reasoned determination about whether the price of PROCSYBI is an "abuse of monopoly power" without considering the economic and clinical context of ultra-rare disease drugs such as PROCYSBI.[16] *Alexion* does not prevent that inquiry or suggest that it falls outside the factors identified in section 85(1). It merely informs the Board that, when it considers the factors under section 85(2), it is to provide reasons as to why it cannot assess "excessiveness" on the factors provided in section 85(1) alone.

21. As *Alexion* informs us, the Board cannot forsake its mandate or ignore the evidence. Rather, it must engage with the evidence, the statutory factors, and the Guidelines and ask whether the sale of PROCYSBI – after its years of investment and development, its satisfaction of a long-felt need, its introduction at a price consistent with the international median price, based on a level of therapeutic improvement borne out in the evidence – is priced excessively. One thing is certain: the maximum non-excessive price cannot be arrived at through Board Staff's three pricing tests, whose singular focus (based on the flawed premise that PROCYSBI is

[16] *Alexion*, para. 54

no better than Cystagon and that the Board's sole purpose is consumer protection) has been to grind the price of PROCSYBI down to the price of Cystagon. That is not patent abuse. That is price control. On this, *Alexion* was abundantly clear.

ALL OF WHICH IS RESPECTFULLY SUBMITTED this 17TH day of September 2021.

Torys LLP
79 Wellington St. W., 30th Floor
Box 270, TD South Tower
Toronto, ON M5K 1N2
Fax: 416.865.7380

Sheila R. Block
Tel: 416.865.7319
sblock@torys.com

Andrew M. Shaughnessy
Tel: 416.865.8171
ashaughnessy@torys.com

Rachael Saab
Tel 416.865.8172
rsaab@torys.com

Stacey Reisman
Tel: 416.865.7537
sreisman@torys.com

Counsel to Respondent,
Horizon Pharma PLC