

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 EXPERT REPORT OF DR. JOEL HAY, PH.D.
(Replying to Responding Expert Report of Mr. Howard Rosen)**

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I, Joel W. Hay, PhD, of the City of Los Angeles in the State of California in the United States of America, provide the following statement of evidence that I propose to present at the hearing of the above referenced proceeding:

I. MANDATE AND ISSUES TO BE ADDRESSED

1. I prepared a report dated September 9, 2019 (“September 2019 Report”) and an addendum report dated July 30, 2020 (“July 2020 Addendum”),¹ in which I concluded (among other things) that, at Board Staff’s Proposed Prices, [REDACTED]

2. Board Staff subsequently retained Mr. Howard Rosen to comment on my economic analysis of cash flows from sales of PROCYSBI in Canada, and to opine on how Board Staff’s Proposed Prices will affect Horizon’s profits.³

3. Accordingly, counsel for Horizon have now asked me to comment on the key areas of disagreement that I have with the Rosen Report.⁴ In addition, given that I have not previously seen the Rosen Report, I have been asked to provide my comments on points of reply.

4. My Expert Reports and the Rosen Report provide background and other information, and this report should be read in conjunction with those reports. For ease of reference, I continue to use the same defined terms as set forth in my Expert Reports. Where Mr. Rosen

¹ I understand that I continue to be bound by the CV and Expert Witness Declaration attached to my September 2019 Report. A complete list of the additional information that I have relied on in preparing this report is listed in Appendix A to this report.

² I have also prepared a report in this matter dated October 9, 2020 (my “October 2020 Responding Report”), where counsel asked me to comment on the Expert Report of Prof. Richard Schwindt. There, I detailed my opinions as to Prof. Schwindt’s analyses of the TCC test, the MIPC Test, and the Moderate Improvement Test, as well as my critique of Prof. Schwindt’s (unsustainable) conclusions that the low prices afforded by those tests would overcompensate Horizon.

Collectively, I refer to my September 2019 Report, July 2020 Addendum Report and October 2020 Responding Report as my “Expert Reports”.

³ Expert Report of Howard Rosen (Secretariat), dated October 6, 2020 (“Rosen Report”), ¶1.1.

⁴ In this report, I comment on the key areas of agreement and disagreement that I have with the Rosen Report. To the extent I do not address an issue raised therein, my non-response should not be taken to mean that I agree or disagree with by Mr. Rosen on that issue.

has used different defined terms for the same concepts, I have specified the defined terms adopted herein.⁵

II. EXECUTIVE SUMMARY

5. As a general matter, nothing in the Rosen Report causes me to change any of the conclusions that I have put forth in my Expert Reports. As discussed below, I disagree with three key areas of Mr. Rosen’s analysis. However, even if one were to accept all of Mr. Rosen’s calculations,⁶ Horizon would not be able to cover the cost of capital it incurred to commercialize PROCYSBI in Canada under Board Staff’s Proposed Prices.

6. My conclusions with respect to Mr. Rosen’s analysis are as follows:

- a) **Mr. Rosen’s “revenue-based” approach to cost allocation institutionalizes “free riding” and thereby disincentivizes the development of rare disease drugs.** In order to ensure that all countries bear their fair share of the cost of commercializing a rare disease drug (like PROCYSBI), the share of development and commercialization costs borne by any country should be independent of inter-country differences in drug prices. Thus, in my earlier reports, [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED] Mr. Rosen’s allocation approach does not ensure that all countries bear their fair share of the cost of commercializing a rare disease drug. Instead, Mr. Rosen allocates these commercialization costs for PROCYSBI using

⁵ In particular, in this report, I use the term Horizon to refer to the entity that Mr. Rosen refers to as the “Horizon Group”. As noted by Mr. Rosen, my analysis of Horizon’s cash flows from PROCYSBI in Canada is, conservatively, undertaken from the perspective of the aggregate profit generated by Horizon globally based on sales of PROCYSBI in Canada. [Rosen Report, ¶¶5.1-5.5.]

⁶ Rosen Report, Figure 3 and Figure 4.

⁷ “Horizon Pharma plc Completes Acquisition of Raptor Pharmaceutical Corp,” Press Release dated October 25, 2016, available at <https://www.sec.gov/Archives/edgar/data/1070698/000119312516745949/d255637dex99a5ii.htm>

⁸ Instead, Mr. Rosen allocates these PROCYSBI investment costs to Canada based on Canada’s share of Horizon’s worldwide revenues from the drug.

a proportion dictated by Horizon's worldwide revenues across all drugs in its portfolio (and not just PROCYSBI).⁹ Mr. Rosen's approach puts Canada in the position of a free rider, allowing Canada to receive its full share of benefits from PROCYSBI while shifting all but a tiny fraction of PROCYSBI's commercialization cost to other countries (such as the U.S.). However, the economics literature has shown that incentives for new-drug development can be undermined when countries use regulation to excessively restrict prices.

- b) **Mr. Rosen and I disagree on particular cost and expense items.** Mr. Rosen appears to view his calculations of other inputs into his financial model, such as cost of goods sold and general and administrative expenses, as a "book-keeping" exercise. In particular, he requires documentation of each expense line item, and if such documentation is unavailable, he ignores the item.¹⁰ Mr. Rosen's deductions are not proper, and he fails to consider the operating realities of a pharmaceutical company. A pharmaceutical company cannot manage its global operations, including its operations in Canada, without incurring many of the costs that are necessary for the head office and business support functions provided by Horizon for PROCYSBI in Canada.
- c) **Mr. Rosen has posited an internal rate of return ("IRR") that confirms that Horizon would not recover its costs.**¹¹ Mr. Rosen calculates the IRRs that Horizon would generate under Board Staff's Proposed Prices.¹² However, he fails to

⁹ See, *e.g.*, Rosen Report, ¶5.69, Schedule 7, Schedule 13.

Mr. Rosen's approach under-allocates PROCYSBI investments costs to Canada because Canada's share of [REDACTED]. This under allocation outcome is entirely predictable because Horizon charges higher prices and thus realizes a disproportionate share of its revenues from the United States. Because Mr. Rosen allocates too small a share of overall drug development costs to Canada, even if he were concerned with setting a price that allowed Horizon to earn a fair rate of return on these costs (which he is not), the amount of cost that Horizon would be allowed to recover would still be insufficient.

¹⁰ See, *e.g.*, Rosen Report, ¶¶5.87-5.88.

¹¹ See Appendix B for more background on the IRR and how it is used to determine if an investment will recover its costs.

¹² Rosen Report, ¶¶6.12-6.19, Figure 4 and Figure 27.

recognize that, at these IRRs, Horizon could not recover its cost of capital. [REDACTED]

[REDACTED] This is well above the IRRs calculated by Mr. Rosen, meaning that Horizon [REDACTED]

[REDACTED]

7. Below, I summarize these major points of disagreement. In the section following I will provide detailed explanations to support my conclusions.

A. Mr. Rosen’s Revenue-Based Approach to Allocation Institutionalizes Free-Riding

8. I was pleased to see that Mr. Rosen adopted my general framework for his financial analysis of PROCYSBI in Canada. Namely, he agrees that the basis for the cost incurred by Horizon to develop and commercialize PROCYSBI includes both (i) the USD\$860.8 million that Horizon paid to acquire PROCYSBI from Raptor (the “Raptor Acquisition Cost”) and (ii) the ongoing R&D Horizon will incur for PROCYSBI.¹³

9. The points of difference between his analysis and mine are depicted in Figure 2 of the Rosen Report, which I have excerpted below for ease of reference. As shown, the major point of disagreement relates to how the Raptor Acquisition Cost and on-going R&D expenses should be allocated to Canada. I will refer to these costs collectively as the “PROCYSBI Commercialization Costs.”¹⁴

¹³ Rosen Report, ¶¶5.68-5.69.

¹⁴ In this report, I focus the discussion on the allocation of the Raptor Acquisition Cost, *i.e.*, the initial commercialization cost, as it makes up the vast majority of total PROCYSBI Commercialization Costs. Specifically, out of the total [REDACTED] allocated by Mr. Rosen, the Raptor Acquisition Cost accounts for [REDACTED]. Similarly, out of the total [REDACTED] I allocated in my September 2019 Report (or [REDACTED] in my July 2020 Addendum), the Raptor Acquisition Cost accounts for [REDACTED] (or [REDACTED]). [See, *e.g.*, Rosen Report, Schedule 13].

Figure 1: Differences Between Mr. Rosen’s and My Financial Analysis



10. As explained in my September 2019 Report, [REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]

11. In my July 2020 Addendum, [REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]

12. In contrast, Mr. Rosen puts forward three distinct “revenue-based” approaches for allocating the PROCYSBI Commercialization Costs to Canada, though he ultimately adopts only one.¹⁵ I briefly summarize each approach, below:

13. **Global Revenue Approach.** Mr. Rosen adopts an unexplained methodology based on Horizon’s global revenues earned from its *entire drug portfolio*. Specifically, he allocates the PROCYSBI Commercialization Costs to Canada using a ratio based on the “average of

¹⁵ Rosen Report, ¶¶5.68-5.69.

revenues reported by Horizon from rest of the world” to “the total revenue earned by Horizon globally” over the past four years (the “**Global Revenue Approach**”).¹⁶

14. This approach does not make economic sense as a measure of sales of PROCYSBI, as it relies on the revenues from Horizon’s entire drug portfolio (12 drugs in total). These non-PROCYSBI revenues are not relevant to the analysis of PROCYSBI. There is no economic rationale for incorporating revenues from drugs other than PROCYSBI, and from jurisdictions other than those in which PROCYSBI is sold. Yet, Mr. Rosen introduces this approach in a single paragraph without any explanation as to why these other revenues are appropriate.

15. ***PROCYSBI Revenue Approach.*** Mr. Rosen spends considerable time discussing an allocation method (that he does not use) that is similarly flawed. This latter method allocates the PROCYSBI Commercialization Costs to Canada using a ratio based on the “revenue reported by Horizon from the sale of PROCYSBI in Canada” to the “total revenue earned by Horizon from the sale of PROCYSBI” (the “**PROCYSBI Revenue Approach**”).¹⁷

16. Mr. Rosen states that he abandoned this approach because he did not have the data in order to make these calculations,¹⁸ a point with which I disagree (in addition to my disagreement with this methodology as an appropriate measure of allocation).

17. In addition to institutionalizing free riding and thereby disincentivizing pharmaceutical companies’ investments in rare disease drugs that benefit Canadian patients, this approach has the perverse impact of penalizing Horizon for bringing PROCYSBI to market in Canada at a price that is significantly lower than its U.S. price.

18. ***KPMG Valuation Approach.*** Before arriving at this secondary technique, Mr. Rosen had suggested that I should have determined the cost of developing and commercializing PROCYSBI in Canada using a KPMG valuation performed at the time that Horizon acquired Raptor (the “**KPMG Valuation Approach**”).¹⁹ This approach would have been wrong. The KPMG valuation projects discounted *future* cash flows that Horizon is expected to earn *after*

¹⁶ Rosen Report, ¶5.69. Note that Mr. Rosen considers these global revenues for the years 2016-2019.

¹⁷ Rosen Report, ¶5.68.

¹⁸ *Ibid.*

¹⁹ Rosen Report, ¶¶5.59-5.67; TOR0000001046.

it commercializes PROCYSBI; it does not relate to or reflect the cost of developing and commercializing PROCYSBI in Canada.

19. In a KPMG valuation performed at the time of the Horizon acquisition of Raptor, [REDACTED]
[REDACTED]
[REDACTED] But, this suggestion is misguided and does not make economic sense for two reasons. First, this valuation is based entirely on future cash flows that Horizon was projected to earn after it commercializes PROCYSBI. It has nothing to do with the upfront costs to develop and commercialize the drug. Mr. Rosen is comparing apples and oranges. Second, I disagree with Mr. Rosen that cystinosis patients in Brazil and Colombia should be included in the calculation. PROCYSBI is neither approved nor commercialized in those countries. Said differently, these Brazil and Colombia patients are not commercial patients.

20. All of these revenue-based approaches allow Canada to act as a “free rider.” Given the much larger market and generally higher prices prevailing in the U.S., Mr. Rosen’s analysis skews the allocation of these upfront costs, leaving but a pittance to be borne by Canada. For example, [REDACTED]
[REDACTED]

21. Moreover, Mr. Rosen’s approach is inconsistent with the study commissioned by the PMPRB on methods of costs allocation that is published on the PMPRB’s website.²¹ Entitled “The Definition of Making and Marketing Costs for Purposes of Section 85(2) of the Patent Act”, this study states that the formula for calculating the “Canadian share of the total cost of developing a drug and bringing it to market” is the “total cost of developing a drug and bringing

²⁰ Rosen Report, Schedule 13.

²¹ McFetridge, D.G., “The Definition of Making and Marketing Costs for Purposes of Section 85(2) of The Patent Act”, Patented Medicines Prices Review Board, available at <http://www.pmprb-cepmb.gc.ca/view.asp?ccid=1071&wbdisable=true>.

it to market is the fully absorbed world-wide average cost per **unit** multiplied by the number of **units** sold in Canada. (emphasis added)”²²

B. Disagreement on Other Cost and Expense Items

22. Mr. Rosen and I disagree on the method for calculating and allocating certain cost and expense items (as depicted in Figure 1, above).²³ We agree on: (i) unit sales and net revenues (*i.e.*, gross revenues, rebates, and copays) from PROCYSBI in Canada; and (ii) the royalties payable by Horizon to the University of California for sale of PROCYSBI in Canada. We also largely agree on the “sales and marketing expenses” and “other cost of sales” incurred by Horizon, though we have some relatively minor differences.²⁴

23. The larger points of disagreement relate to the “Costs of Goods Sold” and “General and Administrative Expenses.”

24. **Cost of Goods Sold.** While Mr. Rosen and I agree on the basis for Horizon’s per-unit cost of goods sold, namely standard costs,²⁵ we disagree on the rate at which these costs grow over time. This point of disagreement accounts for █████ of the difference between our results on the net cash flows from sale of PROCYSBI in Canada. Mr. Rosen’s cites to the Producer Price Index published by the U.S. Bureau of Labor Statistics (the “U.S. PPI”) and argues that the appropriate growth rate for the production costs of PROCYSBI in this case is █████²⁶ I disagree. As a matter of economics, it is not appropriate to apply the U.S. PPI growth rate. First, PROCYSBI is manufactured in Europe, not the U.S.²⁷ Second, this inflation rate encompasses all U.S. industries (including fruit and vegetable canning, grocery stores, and

²² *Ibid.*

²³ See also Rosen Report, Figure 1.

²⁴ The points of disagreement on sales and marketing expenses and other cost of sales account for only █████ of the difference between our results on the net cash flows from PROCYSBI, respectively. [Rosen Report, Figure 2].

²⁵ Rosen Report, ¶¶5.37-5.38.

²⁶ Rosen Report, ¶¶5.32-5.35 and ¶-5.38.

²⁷ Rosen Report, Figure 9 and Figure 10.

electrical power distribution) and does not reflect the cost pressures faced by pharmaceutical producers.

25. *General and Administrative Expenses.* Here, Mr. Rosen agrees with my allocation approach; however, he disagrees that all line items should be included. The point of disagreement accounts for [REDACTED] of the difference in our results on the net cash flows from PROCYSBI. Whereas I included all general and administrative expense line items in my analysis, Mr. Rosen excludes several line items, including those for Horizon's communications, government and public affairs, patient advocacy, corporate development and management, G&A initiatives, facilities and security expenses.²⁸ This approach makes no economic sense and is devoid of common sense. By disregarding these costs entirely, Mr. Rosen ignores the operating realities of a pharmaceutical company. Indeed, this approach suggests that, in Mr. Rosen's view, Horizon (or any pharmaceutical company for that matter) can manage its global operations, including its operations in Canada, without incurring expenses for facilities, communications, public, government and patient relations, and corporate management, among others.

C. Based on Mr. Rosen's Analysis, Horizon Would Not Recover Its Costs Under Board Staff's Proposed Prices

26. Mr. Rosen uses his financial model of the cash flows from sales of PROCYSBI in Canada to calculate Horizon's IRR under Board Staff's Proposed Prices. However, as discussed below, Mr. Rosen's IRR analysis is flawed because it fails to account for all of Horizon's commercialization costs, which include its cost of capital. Mr. Rosen does not compare the resulting IRRs to Horizon's weighted average cost of capital ("WACC") in order to assess Horizon's return on investment from PROCYSBI in Canada. Moreover, Horizon's losses from Mr. Rosen's analysis are understated. I note too that a negative return on investment is inconsistent with the PMPRB's new Guidelines ("New Guidelines").²⁹

²⁸ Rosen Report, ¶¶5.87-5.88.

²⁹ PMPRB Guidelines, Effective January 1, 2020, available at <https://www.canada.ca/content/dam/pmprb-cepmb/documents/legislation/guidelines/PMPRB-Guidelines-en.pdf> ("PMPRB New Guidelines"); PMPRB Draft Guidelines Consultation, available at <https://www.canada.ca/en/patented-medicine-prices-review/services/consultations/draft-guidelines.html>.

27. **Mr. Rosen fails to account for the cost of capital.** Under Mr. Rosen’s analysis of cash flows from PROCYSBI in Canada under Board Staff’s Proposed Prices, Horizon would [REDACTED] [REDACTED] Said differently, even if I were to assume that Mr. Rosen’s financial inputs were correct (which they are not), [REDACTED]

28. Mr. Rosen readily admits that, under the 96% price reduction from the **Same Medicine Comparison Test**, Horizon would earn – on an undiscounted basis – [REDACTED] [REDACTED] In the cases of the 80% and 71% prices reductions under Board Staff’s **Market Share Comparison Test** and **Premium Comparison Test**, Mr. Rosen calculates that Horizon would earn – on an undiscounted basis – [REDACTED] [REDACTED] He translates these latter amounts into IRRs of [REDACTED]³² However, these amounts do not fully reflect Horizon’s costs because they do not incorporate its cost of capital.³³ When the cost of capital is considered, as it should be, [REDACTED] [REDACTED]

29. **Mr. Rosen’s IRR analysis is flawed.** Mr. Rosen calculates Horizon’s IRR from PROCYSBI at Board Staff’s Proposed Prices. However, the IRR cannot be used on its own to determine whether a company will, in fact, earn a profit on investment. In order to make such a determination, the IRR must be compared to the threshold rate of return that a company must earn for an investment to be profitable; this threshold rate is commonly referred to as the

³⁰ Rosen Report, ¶1.22, Figure 3, ¶¶6.1-6.11 and Figure 25.

³¹ *Ibid.*

³² Rosen Report, ¶1.23, Figure 4, ¶¶6.2-6.19 and Figure 27.

³³ It is important to put these amounts varying from [REDACTED] calculated by Mr. Rosen in the proper context. As noted by Mr. Rosen himself, they represent the total, undiscounted cash flows Horizon would generate from PROCYSBI in Canada during the period between Horizon’s acquisition of Raptor in October 2016 through to [REDACTED] – a period of approximately [REDACTED]. Said differently, Mr. Rosen’s calculations show that Horizon would generate average *annual* cash flows of between – [REDACTED] per year and [REDACTED] [REDACTED] under Board Staff’s Proposed Prices (see Figure 6 below). Note too, that (without discounting) this puts equal weight on the value of cash flows earned in [REDACTED] as those incurred today. [REDACTED]

Weighted Average Cost of Capital (“WACC”).³⁴ Based on my analysis, the relevant WACC rate for Horizon at the time of the Raptor acquisition is [REDACTED]³⁵ Mr. Rosen’s calculated IRRs are well below this rate – meaning that, even if one were to assume that Mr. Rosen’s financial inputs were appropriate (which they are not), this comparison demonstrates that Horizon would be [REDACTED]

30. ***Horizon’s losses are understated.*** It is also important to note that the financial models Mr. Rosen and I have used may understate the losses that Horizon would incur for PROCYSBI in Canada under Board Staff’s Proposed Prices. First, Board Staff’s proposed price reductions are greater than those used in both of our analyses. Board Staff seek a reduction in the ex-factory price of PROCYSBI of between 96% and 98% under the Same Medicine Comparison Test, 80% and 92% under the Market Share Comparison Test, and 71% and 73% under the Premium Comparison Test.³⁶ To be conservative, Mr. Rosen and I have used the lower bound of each price range.³⁷ Had each of us used the higher range of Board Staff’s proposed reductions, Horizon would incur even larger losses than those calculated above. Second, for the purpose of forecasting the sales volumes of PROCYSBI in Canada, I was instructed to assume that Horizon would have market exclusivity in Canada through to the expiry of PROCYSBI’s patents in [REDACTED]. However, if Horizon were to lose market exclusivity (for example, as a result of entry by a generic manufacturer) during this period, its sales volume would be expected to decrease substantially.

31. ***A negative rate of return jeopardizes rare-disease drug development.*** A negative return on investment is inconsistent with an economically viable enterprise, especially one focused on serving the small patient populations associated with rare diseases. It is also

³⁴ See Appendix B.

³⁵ See Appendix C for details of my analysis of the relevant WACC.

³⁶ See, e.g., Figure from my September 2019 Report.

³⁷ See, e.g., Rosen Report, Figure 3.

³⁸ This assumption is also embedded in Mr. Rosen’s analysis as he has “adopted [my] assumptions regarding sales volume and prices in [his] analysis and calculations. [Rosen Report, Figure 1.]”

inconsistent with the PMPRB’s new Guidelines,³⁹ which incorporate the principle that drugs for rare diseases need to generate a reasonable return (via a relatively higher price) in order to incentivize development.⁴⁰ As explained in my September 2019 Report, the price charged by a pharmaceutical manufacturer of a rare-disease drug, such as Horizon with PROCYSBI, must provide the manufacturer with the opportunity to recover the costs it incurred to commercialize the drug and generate a return on investment over a very small patient base. Indeed, the economics expert retained by Board Staff – Prof. Schwindt – agrees that an appropriate price must include “a normal return to entrepreneurial effort (*i.e.*, profits)”.⁴¹ Accordingly, restrictions on prices – such as those proposed by Board Staff – that prevent manufacturers from recovering the costs of capital associated with commercializing new rare disease drugs can be expected to have a negative impact on future investment in the development and provision of these drugs in Canada.

32. Below, I will discuss each of these summary conclusions in greater detail.

III. MR. ROSEN’S REVENUE-BASED APPROACH TO ALLOCATION INSTITUTIONALIZES FREE-RIDING, THEREBY DISINCENTIVIZING THE DEVELOPMENT OF RARE DISEASE DRUGS

A. My Approach to Allocation Is Based on Unit Sales

33. Mr. Rosen adopts my general framework for his financial analysis of PROCYSBI in Canada, as well as many of the inputs I used, including the basis for the PROCYSBI Commercialization Costs.⁴² However, as stated, we disagree about how this cost should be allocated to Canada.

³⁹ PMPRB Guidelines, Effective January 1, 2020, available at <https://www.canada.ca/content/dam/pmprb-cepmb/documents/legislation/guidelines/PMPRB-Guidelines-en.pdf> (“PMPRB New Guidelines”).

⁴⁰ PMPRB Draft Guidelines Consultation, available at <https://www.canada.ca/en/patented-medicine-prices-review/services/consultations/draft-guidelines.html>.

⁴¹ Schwindt Report, pp. 2-3.

⁴² See, *e.g.*, Rosen Report, Figure 1.

34. As reflected in my Expert Reports, which dealt with the problem of funding development for rare disease drugs,⁴³ in order to ensure that all similarly-situated countries bear their fair share of the cost of commercializing rare disease drugs, the share of commercialization costs borne by any country should be independent of inter-country differences in drug prices.⁴⁴ Instead, this share should be dictated by a figure that is directly proportional to the benefits that the medicine provides to patients in each country.

35. [REDACTED]

⁴³ See, e.g., Tambuyzer, E. (2010). Rare Diseases, Orphan Drugs and their Regulation: Questions and Misconceptions. *Nature Reviews Drug Discovery*, 9(12): 921-929; Hollis, A. (2006). Drugs for Rare Diseases: Paying for Innovation. In C.M. Beach et al. (eds.) *Health Services Restructuring in Canada: New Evidence and New Directions*: 155-177. Queen's School of Policy Studies; Rollet, P. et al. (2013). Sustainable Rare Diseases Business and Drug Access: No Time for Misconceptions. *Orphanet Journal of Rare Diseases*, 8(1): 109-118.

⁴⁴ Note that External Reference Based Pricing is not weighted or skewed by market sizes.

⁴⁵ September 2019 Report, Appendix F, paras. 22-23.

⁴⁶ Since the amount of drug each patient needs for therapy is objectively determined based on a clinical dosing regimen, the amount of product that Horizon could expect to supply to cystinosis patients in each jurisdiction should be proportional to the total number of patient in that jurisdiction.

⁴⁷ An attractive feature of this approach is that it would tend to reflect the demand for PROCYSBI over the "long run". In particular, I understand that the population of nephropathic cystinosis patients has been relatively stable over time, as would be consistent with a disease caused by a genetic mutation. Accordingly, one would expect that the ratio of cystinosis patients in Canada to total patients worldwide would be proportional to the ratio of PROCYSBI sales in Canada to total worldwide sales over the long run. Said differently, unlike the approach used by Mr. Rosen, it would not be dependent on the time period considered. [Langman Report, ¶¶34-44; Elmonem, MA. et al. (2016). Cystinosis: A Review. *Orphanet Journal of Rare Diseases*, 11(47), 1-17.]

⁴⁸ [REDACTED]

36.

[REDACTED]

B. Mr. Rosen’s “Revenue-Based” Approach to Allocation is Flawed

37. Mr. Rosen allocates the PROCYSBI Commercialization Costs to Canada using the Global Revenues Approach, described above.^{50,51} [REDACTED]

[REDACTED]

38. Although Mr. Rosen ultimately adopts the Global Revenues Approach, I note at the outset that he puts forward two other distinct “revenue-based” approaches (*i.e.*, the PROCYSBI Revenue Approach⁵³ and KPMG Valuation Approach⁵⁴). All three methods depend on the prices charged for, and thus revenues earned from, PROCYSBI in other countries. As explained below, these approaches institutionalize free riding, thereby disincentivizing the

[REDACTED]

⁴⁹ Hay July 2019 Addendum, ¶2 and Figure 1.

⁵⁰ Rosen Report, Schedule 7 and Schedule 13.

⁵¹ Rosen Report, Figure 1 and ¶¶5.69 and Schedule 13.

⁵² [REDACTED]

⁵³ Rosen Report, ¶5.68.

⁵⁴ Rosen Report, ¶¶5.59-5.67.

development of new rare disease drugs. Moreover, they are inconsistent with the cost allocation formula from a study commissioned by the PMPRB “to assist [it] in developing criteria which will enable it to define the ‘making’ and ‘marketing’ costs of patented medicines for purposes of Section 85(2) of the Patent Act.”⁵⁵

(i) A Revenue-Based Approach Institutionalizes Free Riding and Thereby Disincentivizes the Development of New Rare-Disease Drugs

39. In contrast to the approach reflected in my Expert Reports, Mr. Rosen’s revenue-based approaches depend on the price and market share of PROCYSBI in other countries. In particular, Mr. Rosen argues that, “[s]ince almost all of the revenues earned by Horizon are generated from the US, the Raptor Acquisition Cost and ongoing R&D expenses should be allocated on a pro-rata basis according to the revenues generated by each region.”⁵⁶ Given higher U.S. drug prices, adoption of Mr. Rosen’s method would put Canada in the position of a free rider, allowing Canada to receive its full share of benefits from PROCYSBI while shifting all but a tiny fraction of PROCYSBI Commercialization Costs to other countries (such as the US).⁵⁷ In effect, Mr. Rosen is advocating for Canada, and indeed the rest of the world, to free ride on the costs borne by U.S. patients.

40. The obvious problem with the free riding approach promoted by Mr. Rosen is that, if all countries in the world tried to do it simultaneously, there would be no country left to cover the costs that pharmaceutical companies incur during the drug development process. In fact, this type of free riding constitutes a “market failure” that could disincentivize research and development investment into new drugs, particularly drugs for rare diseases (like PROCYSBI for cystinosis). Indeed, the economics literature has shown that incentives for new-drug development can be undermined in countries that use regulation to excessively restrict prices (and with the overall impact of those regulations on the affordability of pharmaceuticals being

⁵⁵ McFetridge, D.G., “The Definition of Making and Marketing Costs for Purposes of Section 85(2) of The Patent Act”, Patented Medicines Prices Review Board, available at <http://www.pmprb-cepmb.gc.ca/view.asp?ccid=1071&wbdisable=true>.

⁵⁶ Rosen Report, ¶5.66.

⁵⁷ Indeed, if prices were the same across countries, the common price would cancel itself out of the calculation (as it would appear in both the numerator and denominator of the ratio). The result would then be an allocation based on relative unit sales, as I have recommended.

ambiguous).⁵⁸ At the international level, free riding effects can result in price instability that leads to launch delays and unwillingness of drug manufactures to launch in countries that regulate prices below market levels.⁵⁹

41. As discussed in my September 2019 Report, the social welfare loss associated with such free riding stems from the loss of drug products that would no longer be developed due to manufacturer disincentives. Economic studies have shown that the societal returns on pharmaceutical development are large, especially for drugs for rare diseases.⁶⁰ For example, a study by Lichtenberg and Waldfogel (2003) investigated the relationship between the health benefits to patients with rare diseases and the increased R&D incentives stemming from the passage of the U.S. Orphan Drug Act. The study found that availability of novel therapies for rare diseases had a statistically significant effect on the longevity of people suffering from these conditions.⁶¹ Such benefits for Canada would be jeopardized if free riding were institutionalized through adoption of Mr. Rosen's revenue-based allocation approaches.

⁵⁸ See, e.g., Scherer, F.M. (2001). The Link Between Gross Profitability and Pharmaceutical R&D Spending. *Health Affairs*, 20(5), 216-220; Kessler, D.P. (2004). The Effects of Pharmaceutical Price Controls on the Cost and Quality of Medical Care: A Review of the Empirical Literature. *Mimeo*; Vernon, J.A. (2005). Examining the Link Between Price Regulation and Pharmaceutical R&D Investment. *Health Economics*, 14(1), 1-16; Giaccotto, C. et al. (2005). Drug Prices and Research and Development Investment Behavior in the Pharmaceutical Industry. *The Journal of Law and Economics*, 48(1), 195-214; Danzon, P.M., et al. (2005). The Impact of Price Regulation on the Launch Delay of New Drugs—Evidence from Twenty-Five Major Markets in the 1990s. *Health Economics*, 14(3), 269-292; Golec, J.H., and Vernon, J.A. (2006). European Pharmaceutical Price Regulation, Firm Profitability, and R&D Spending. National Bureau of Economic Research, No. w12676; Eger, S. and Mahlich, J.C. (2014). Pharmaceutical Regulation in Europe and its Impact on Corporate R&D. *Health Economics Review*, 4(23), 1-9; Kanavos, P., et al. (2017). *The Impact of External Reference Pricing within and across Countries*. London: London School of Economics, pp. 15-21, 26-28 and 39-44; Pertile, P. et al. (2018). Free-Riding in Pharmaceutical Price Regulation: Theory and Evidence. *Mimeo*, Department of Economics, University of York. No. 18/04.

⁵⁹ *Ibid.*

⁶⁰ *Ibid.*

⁶¹ Lichtenberg, F.R. and Waldfogel, J. (2003). Does misery love company? Evidence from pharmaceutical markets before and after the Orphan Drug Act. National Bureau of Economic Research, No. w9750.

Specifically, Lichtenberg and Waldfogel (2003) found that the percent of individuals dying young for relatively rare illnesses fell from by 6 percentage points between 1979 and 1998, whereas the percent of patients dying young from more common disease conditions had fallen only by 2 percentage points.

42. In short, relying on the ability to “free ride” on the drug-commercialization costs borne by U.S. patients can be expected to have a negative impact on companies’ continued investment in bringing new rare disease drugs like PROCYSBI to Canada.

(ii) A Revenue-Based Approach is Inconsistent with the Formula Published in the PMPRB Commissioned Study on the Definition of Costs for Purposes of Section 85(2) of the Patent Act

43. Mr. Rosen’s method is inconsistent with the above mentioned study commissioned by the PMPRB on methods of costs allocation for pharmaceuticals in Canada, entitled “The Definition of Making and Marketing Costs for Purposes of Section 85(2) of the Patent Act,” which is published on the PMPRB’s website.⁶² This study states that the formula for calculating the “Canadian share of the total cost of developing a drug and bringing it to market” is the “total cost of developing a drug and bringing it to market is the fully absorbed world-wide average cost per unit multiplied by the number of units sold in Canada,” as illustrated by the following formula:⁶³

$$CDN\ SHARE = TC \left(\frac{q^C}{Q} \right)$$

Ratio of
Canada Units
to
Worldwide Units

44. This formula states that the total cost of commercializing the drug (*i.e.*, “*TC*”) is to be allocated to Canada (*i.e.*, “*CDN SHARE*”) based on the ratio of the drug’s unit sales in Canada (*i.e.*, “*q^C*”) to the total worldwide unit sales of the drug (*i.e.*, “*Q*”).

45. [REDACTED]

⁶⁴ This method is consistent with the requirement under section 85(3) of the *Patent*

⁶² McFetridge, D.G., “The Definition of Making and Marketing Costs for Purposes of Section 85(2) of The Patent Act”, Patented Medicines Prices Review Board, available at <http://www.pmprb-cepmb.gc.ca/view.asp?ccid=1071&wbdisable=true>.

⁶³ *Ibid*, see footnote 28. Here, “*TC* is (worldwide) total product-specific cost, *Q* is worldwide sales (in units) and *q^C* is Canadian sales.”

⁶⁴ Hay July 2019 Addendum, ¶2.

Act that commercialization costs be based on “the ratio of sales by the patentee in Canada of that medicine to total world sales”.⁶⁵

46. As a matter of economics, this approach to allocating the commercialization costs of a new drug is reasonable, as it is based only on the quantities demanded of the drug (and not prices), and thus would reflect the underlying demand for the drug – irrespective of prices. Mr. Rosen’s method is inconsistent with this formula.

C. Mr. Rosen’s Allocation Methodologies are Fundamentally Flawed

47. The criticisms described above apply to all three methodologies discussed in the Rosen Report. There are, however, additional flaws inherent within each methodology. I discuss these below.

(i) Global Revenues Approach

48. Although Mr. Rosen discusses three methodologies, he ultimately adopts the Global Revenues Approach – an unexplained methodology based on Horizon’s global revenues from its entire drug portfolio. [REDACTED]

[REDACTED]

[REDACTED]⁶⁶

49. However, this method is arbitrary. Mr. Rosen’s methodology is based on the revenues from Horizons’ entire drug portfolio. Mr. Rosen allocates PROCYSBI Commercialization Costs to Canada using a ratio based on the “average of revenues reported by Horizon from rest of the world” across all Horizon products to “the total revenue earned by Horizon globally”

⁶⁵ Rosen Report, ¶5.58 (internal citations omitted).

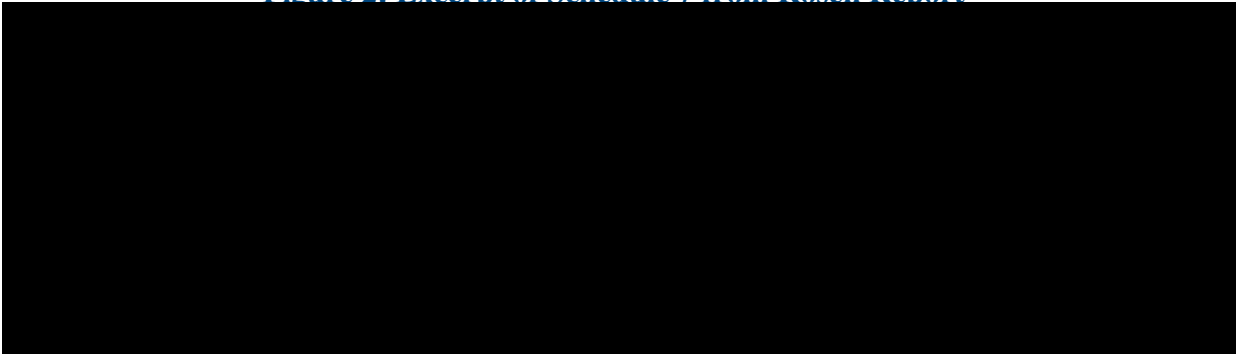
⁶⁶ Rosen Report, Figure 1 and ¶¶5.69 and Schedule 13.

⁶⁷ [REDACTED]

over the past four years.”⁶⁸ But, the revenues from other drug products in other countries are not relevant to the analysis of PROCYSBI. Yet, Mr. Rosen introduces this approach in a single paragraph and without any explanation as to why these other revenues should be considered.⁶⁹ Thus, there is no apparent economic rationale for incorporating revenues from drugs other than PROCYSBI and from jurisdictions other than those in which PROCYSBI is sold.⁷⁰

50. Figure 2, below, taken as an excerpt from the Rosen Report, shows the “Net Revenues from Rest of World” and “Net Revenues of Horizon Group” Mr. Rosen uses for his calculations. He takes the average of these amounts over a four-year period (2016-2019) to arrive at his conclusion that Canada should bear █████ of the PROCYSBI Commercialization Costs.⁷¹

Figure 2: Excerpt of Schedule 7 from Rosen Report



51. These revenue amounts relied on by Mr. Rosen are taken from Horizon’s annual financial report, an excerpt of which for 2016 is provided in Figure 3 below. As shown in Figure 3, the revenues used by Mr. Rosen include 11 other Horizon drugs. These other Horizon drugs cover a range of ailments – many of which affect broad-based patient populations,

⁶⁸ See Rosen Schedule 7 as compared to *e.g.*, Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2016, pp. 4-8; Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2019, pp. 4-11.

⁶⁹ Rosen Report, ¶5.69.

⁷⁰ Rosen Report, Schedule 7; Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2016, F-35 to F-36; Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2017, F-32; Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2018, F-29 to F31; Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2019, F-29 to F31.

⁷¹ For example, in Figure 2 above, Mr. Rosen shows “Net Revenues from Rest of World” in 2016 of █████ and “Net Revenues of Horizon Group” of █████ resulting in a ratio of █████.

including, for example, pain from osteoarthritis of the knee (PENNSAID), gout (KRYSTEXXA), inflammation from certain allergic, skin, stomach and intestinal, blood, eye, nerve, kidney, breathing, rheumatologic, and specific infectious diseases or conditions (RAYOS), and cystic fibrosis (QUINSAIR).⁷²

Figure 3: Excerpt of Horizon Annual Report for 2016

The following table presents a summary of total net revenues by medicine (in thousands):

	Year Ended December 31,		
	2016	2015	2014
PENNSAID 2%	\$ 304,433	\$ 147,010	\$ —
DUEXIS	173,728	190,357	83,243
RAVICTI	151,532	86,875	—
VIMOVO	121,315	166,672	162,954
ACTIMMUNE	104,624	107,444	25,251
KRYSTEXXA	91,102	—	—
RAYOS	47,356	40,329	19,020
PROCYSBI	25,268	—	—
BUPHENYL	16,879	13,458	—
MIGERGOT	4,651	—	—
LODOTRA	4,193	4,899	6,487
QUINSAIR	1,039	—	—
Litigation settlement	(65,000)	—	—
Total net revenues	\$ 981,120	\$ 757,044	\$ 296,955

The following table presents a summary of total net revenues by geography (in thousands):

	Year Ended December 31,		
	2016	2015	2014
United States	\$ 964,041	\$ 744,036	\$ 290,396
Rest of world	17,079	13,008	6,559
Total net revenues	\$ 981,120	\$ 757,044	\$ 296,955

Source: Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2016, F-35 to F-36.

52. As stated above, the revenues from other drug products in other countries are not relevant to the analysis of PROCYSBI, and it is inappropriate to incorporate revenues from drugs other than PROCYSBI (a rare-disease drug) and from jurisdictions other than those in which PROCYSBI is sold.⁷³

⁷² PENNSAID website, available at <https://www.pennsaid.com>; KRYSTEXXA website, available at <https://www.krystexxa.com>; RAYOS website, available at <https://www.rayosrx.com>; QUINSAIR Product Monograph, available at <https://www.hzndocs.com/QUINSAIR-Product-Monograph-English.pdf>.

⁷³ I note too that Mr. Rosen's calculation includes revenues from various drugs which are at very different points in their respective product life cycles. For example, PENNSAID was first approved for sale in the US in 2009, and DUEXIS was first approved for sale in 2011. Accordingly, these two drugs would have long achieved their "steady state" sales, *i.e.*, reached mature market position" – for example, they accounted for almost 50% of Horizon's total revenues in 2016. Conversely, PROCYSBI did not receive Health Canada approval until June 2017, and I understand it was not

(ii) PROCYSBI Revenue Approach

53. Mr. Rosen claims that the most appropriate method for allocating the PROCYSBI Commercialization Costs to Canada would be “based on the ratio of revenue reported by Horizon from the sale of PROCYSBI in Canada to the total revenue earned by Horizon from the sale of PROCYSBI.”⁷⁴ However, Mr. Rosen did not apply this method because he claimed he did not have the necessary data.⁷⁵ That is incorrect. Indeed, this statement is contradicted by Mr. Rosen, himself, when he cites to the supporting document that I had used for my September 2019 Report, which provides the necessary inputs for Mr. Rosen’s calculation.⁷⁶

54. In any event, this approach suffers from the criticisms discussed above: (i) it will disincentivize the development of rare disease drugs like PROCYSBI by effectively institutionalizing Canadian free riding; and (ii) it is inconsistent with the formula from the PMPRB commissioned study on methods of costs allocation for pharmaceuticals in Canada that is published on the PMPRB’s website.⁷⁷ Moreover, Mr. Rosen’s approach has the perverse impact of penalizing Horizon for bringing PROCYSBI to market in Canada at a price that is

listed on Canadian provincial formularies until after July 2018 (when it signed its agreement with the pCPA). It is inappropriate to compare drugs at different points in the product lifecycle. Given the time that it takes for a new drug product to “ramp-up” and penetrate the market to its full potential, it is inappropriate to rely on revenues from mature drug products in this case, given that PROCYSBI in Canada was in its infancy during the period considered by Mr. Rosen. The evolution in growth from the period of launch is reflected in Schedule 7 of the Rosen Report (above): Mr. Rosen’s own calculation shows that PROCYSBI’s share of total Horizon revenues grew by a factor of 5x – from 2.6% in 2016 to 12.5% in 2019. [U.S. FDA Approval Letter, PENNSAID NDA, available at https://www.accessdata.fda.gov/drugsatfda_docs/appletter/2009/020947s000ltr.pdf; U.S. FDA Approval Letter, DUEXIS NDA, available at https://www.accessdata.fda.gov/drugsatfda_docs/appletter/2011/022519s000ltr.pdf; PROCYSBI, Health Canada NOC Database, available at <https://health-products.canada.ca/noc-ac/info.do?lang=en&no=19408>.]

⁷⁴ Rosen Report, ¶5.68.

⁷⁵ Specifically, he states that the “disclosures in Horizon’s financial statements do not provide the breakdown of revenues from the sale of PROCYSBI in Canada and across the rest of the world from 2016 to 2019.” [Rosen Report, ¶5.68].

⁷⁶ Specifically, Mr. Rosen cites to the TOR0000001057, which provides the necessary inputs for Mr. Rosen’s calculation in the tab named “Rev & COGS BPC Pull”. See also Figure 4. [Rosen Report, Appendix 3 – Scope of Review.]

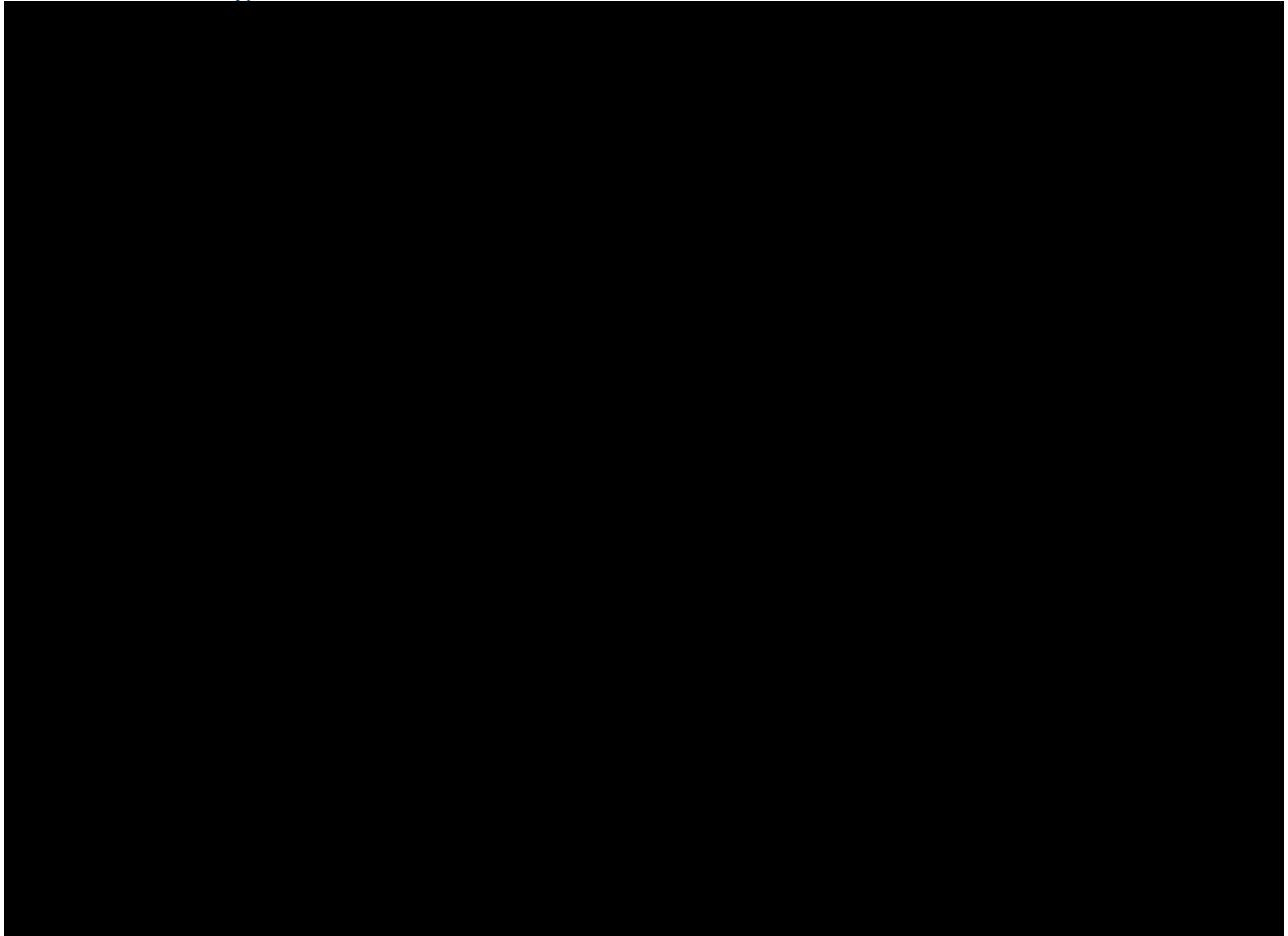
⁷⁷ McFetridge, D.G., “The Definition of Making and Marketing Costs for Purposes of Section 85(2) of The Patent Act”, Patented Medicines Prices Review Board, available at <http://www.pmprb-cepmb.gc.ca/view.asp?ccid=1071&wbdisable=true>.

significantly lower than its U.S. price. This perverse impact is illustrated in the following examples:

- Mr. Rosen and I both calculate that, under its current ex-factory price, Horizon would generate net revenues of approximately [REDACTED] from sales of PROCYSBI in Canada in 2019. Horizon's Annual Report for 2019 reports worldwide net revenues from PROCYSBI of USD \$161.9 million. Accordingly, Mr. Rosen's proposed allocation approach would result in [REDACTED] of PROCYSBI Commercialization Costs being allocated to Canada, as shown in the left column of Figure 4, below. This results in an allocation percentage that is substantially greater than what Mr. Rosen calculated in his report [REDACTED].
- If Horizon had launched PROCYSBI in Canada at the same price for which it sells PROCYSBI in the U.S. (*i.e.*, a price of at least \$1.4045 per mg)⁷⁸, Horizon would have generated net revenues of approximately [REDACTED] from sales of PROCYSBI in Canada in 2019. Given these sales, Mr. Rosen's proposed allocation approach would result in [REDACTED] of the cost to commercialize PROCYSBI being allocated to Canada. This results in an allocation that is substantially greater than what I calculated in my September 2019 Report [REDACTED] and July 2019 Report [REDACTED].

⁷⁸ Statement of Allegations of Board Staff, ¶31.

Figure 4: PROCYSBI Revenues in Canada under the U.S. Price



(iii) KMPG Valuation Approach

55. In addition to the approaches described above, Mr. Rosen suggests that I should have determined the cost of developing and commercializing PROCYSBI in Canada using a KPMG valuation performed at the time that Horizon acquired Raptor.⁷⁹ [REDACTED]

[REDACTED]⁸⁰ Mr. Rosen proceeds to assert that this [REDACTED] value calculation should be allocated among three countries that he (incorrectly) claims comprise the Ex-US and Ex-EMA region – Brazil, Colombia, and Canada. Using this allocation method, Mr. Rosen allocates [REDACTED] to Canada.⁸¹ This suggestion is misguided and does not make economic sense.

⁷⁹ Rosen Report, ¶¶5.59-5.67.

⁸⁰ TOR0000001046.

⁸¹ Rosen Report, ¶5.64.

56. As an initial matter, I disagree that KPMG's [REDACTED] value for PROCYSBI Ex-US and Ex-EMEA is an appropriate basis to determine the cost of PROCYSBI. This valuation has nothing to do with the *upfront* cost incurred by Horizon to acquire PROCYSBI. Rather, this valuation is based entirely on the discounted *future* cash flows that Horizon is projected to earn *after* it commercializes PROCYSBI.⁸² This is the apples and oranges point I made in the summary section above. By definition, the [REDACTED] valuation does not reflect the cost of developing and commercializing PROCYSBI, and it would therefore be inappropriate to use this as a basis for allocating costs.

57. I also disagree with Mr. Rosen's assertion that the number of cystinosis patients in Brazil and Colombia is a relevant metric for allocating costs. It is my understanding that the total prospective market for PROCYSBI is Canada, the U.S., and Europe. For example, in its press releases announcing marketing approvals for PROCYSBI, Horizon cites only to the populations of 800 cystinosis patients in Europe, 500 in the U.S. and 100 in Canada (but makes no mention of Brazil or Colombia).⁸³

58. Based on my discussion with Horizon management, I understand that patients in Brazil and Colombia who receive PROCYSBI are not "commercial patients." PROCYSBI is not approved in either Brazil or Colombia. Horizon has not received (and has never sought) marketing approval for PROCYSBI in Brazil or Colombia. Rather, Horizon had a research site in Brazil in connection with PROCYSBI's clinical trials, following which "participants were

⁸² Mr. Rosen cites to a set of lecture notes from Aswath Damodaran, a financial economist, to support his proposed allocation methodology. However, these lecture notes are on the topic of valuation – they have nothing to do with allocating the *upfront* cost for an investment project, and instead discuss valuing the *future* cash flows that would be generated *after* the investment has been made. Rosen Report, footnote 126; Damodaran, A., "The Value of Intangibles", available at <http://people.stern.nyu.edu/adamodar/pdfiles/ovhds/dam2ed/intangibles.pdf>.

⁸³ "Raptor Pharmaceutical Receives Marketing Authorization for PROCYSBI in European Union", Press Release dated September 12, 2013, available at https://www.sec.gov/Archives/edgar/data/1070698/000107069813000073/rptp_pr091213_euprocysapprvl.htm; "Raptor Pharmaceutical's PROCYSBI Receives FDA Approval for the Treatment of Nephropathic Cystinosis", Press Release dated April 30, 2013, available at https://www.sec.gov/Archives/edgar/data/1070698/000107069813000018/rptp_prrls_043013.htm; "Raptor's PROCYSBI® New Drug Submission Accepted by Health Canada with Priority Review," Press Release dated March 26, 2016, available at <https://www.globenewswire.com/news-release/2016/03/21/821869/0/en/Raptor-s-PROCYSBI-New-Drug-Submission-Accepted-by-Health-Canada-with-Priority-Review.html>.

eligible to transition to a post-study drug supply program, and continue to receive the drug at no personal cost.”⁸⁴

59. Accordingly, the patients in Brazil and Colombia cited to by Mr. Rosen should not be included in a calculation of “the ratio of sales by the patentee in Canada of that medicine to total world sales.”⁸⁵ [REDACTED]

60. [REDACTED]

IV. OTHER AREAS OF DISAGREEMENT: COST AND EXPENSE ITEMS

61. As discussed above, the main point of disagreement is about the proper method for allocating the PROCYSBI Commercialization Costs to Canada. Mr. Rosen adopts my general framework for his financial analysis of PROCYSBI in Canada, as well as many of the inputs I used. Specifically, he agrees with my conclusions as to: the unit sales and net revenues (including the gross revenues, rebates, and copays) from PROCYSBI in Canada,⁸⁶ as well as the royalties payable by Horizon to the University of California on sales of PROCYSBI in Canada.⁸⁷

⁸⁴ Safety/Effectiveness Study of Cysteamine Bitartrate Delayed-release Capsules (RP103) in Cysteamine Treatment Naive Patients with Cystinosis, available at <https://clinicaltrials.gov/ct2/show/NCT01744782>.

I understand that one or more sites located in Brazil participated in a PROCYSBI clinical trial sponsored by Raptor Pharmaceuticals, which is now part of Horizon, following which participants were eligible to transition to a post-study drug supply program, and continue to receive the drug at no personal cost. Under the conditions placed on Horizon by the Brazilian government for these clinical trials, Horizon is obligated to continue providing PROCYSBI to these patients on an ongoing basis.

⁸⁵ Rosen Report, ¶5.58 (internal citations omitted).

⁸⁶ Rosen Report, Figure 1 and ¶¶5.20-5.27.

⁸⁷ Rosen Report, Figure 1 and ¶¶5.39-5.40.

62. However, we disagree on the method for calculating and allocating certain cost and expense items, as discussed in detail below.

A. Horizon as a Global Entity

63. Before turning to the larger differences between Mr. Rosen’s and my approach to these additional cost and expense elements, it is important to understand the perspective from which each of us have undertaken our analysis. This is important because Horizon “does not segment its business for internal reporting” in the ordinary course of business.⁸⁸ In other words, for items incurred at the company level, Horizon does not track expenses separately for PROCYSBI sold in Canada. Thus, Mr. Rosen and I have had to allocate such expenses for our analyses.

64. My analysis considers the aggregate profit generated by Horizon globally based on sales of PROCYSBI in Canada.⁸⁹ This approach is conservative because it includes the total cash flows generated by Horizon on sales of PROCYSBI in Canada (*i.e.*, both the cash flows from the Horizon Group manufacturing and providing (at a transfer price) PROCYSBI to Horizon Canada, and then the resulting cash flows from Horizon Canada reselling PROCYSBI to patients in Canada). In contrast, Mr. Rosen’s analysis is “from the perspective of Horizon’s business globally” and “from the perspective from Horizon Canada”.⁹⁰

65. As a matter of economics, Mr. Rosen’s analysis “from the perspective from Horizon Canada” does not make any sense – Horizon Canada was established for the purpose of marketing of PROCYSBI in Canada. Mr. Rosen himself acknowledges that “Horizon only maintains a sales and marketing team in Canada” and that “Horizon Canada continu[es] to have minimal involvement in the actual stocking and movement of goods.”⁹¹

⁸⁸ See, *e.g.*, Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2016, F-35.

⁸⁹ Rosen Report, ¶¶5.1-5.5.

⁹⁰ See, *e.g.*, Rosen Report, ¶¶1.22, 6.1 and 6.21.

In particular Mr. Rosen only includes “other cost of sales” and general and administrative expenses in his analysis “from the perspective of Horizon’s business globally”, but not “from the perspective from Horizon Canada”.

⁹¹ Rosen Report, ¶¶5.75-5.76 and ¶5.87.

66. In its financial statements, Horizon emphasizes that it “operates as one segment” and that it “does not segment its business for internal reporting.”⁹² Horizon – as the parent company of Horizon Group – is the operator and administrator of all Horizon Group operations. In the context of this case, this means that it is ultimately Horizon – and not Horizon Canada – that commercialized PROCYSBI in Canada.⁹³ In short, Horizon Canada does not function independently from its parent, Horizon.

67. Accordingly, Mr. Rosen’s analysis “from the perspective from Horizon Canada” is irrelevant. The decision-making entity – that is, the entity that controls the manufacture of PROCYSBI and its in Canada – is Horizon. As a matter of economics, only Horizon’s “perspective” is relevant in assessing the return on investment from PROCYSBI in Canada.

B. Disagreement on Specific Cost Items

68. As stated above, Mr. Rosen and I disagree on the method for calculating and allocating certain cost and expense items. While we largely agree on the “sales and marketing expenses” and “other cost of sales” incurred by Horizon, there are minor areas of difference.^{94,95}

⁹² See, e.g., Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2016, F-35.

⁹³ For example, the Health Canada Notice of Compliance for PROCYSBI was issued to Horizon Pharma Ireland, Ltd., which I understand is the wholly owned and controlled subsidiary of Horizon responsible for its acquisition and development of intellectual property relating to pharmaceuticals. [Health Canada NOC Database, PROCYSBI, available at <https://health-products.canada.ca/noc-ac/info.do?lang=en&no=19408>.]

⁹⁴ On the issue of sales and marketing expenses, which relate to the costs Horizon incurs for its sales force and promotion of PROCYSBI to generate sales in Canada, the point of disagreement accounts for only [REDACTED] of the difference between our results on the net cash flows from PROCYSBI. In particular, our difference is based on a [REDACTED] I made to the amount of such expenses incurred by Horizon in 2017, based on my discussions with Horizon business representatives. Mr. Rosen disagrees with this adjustment because, according to Mr. Rosen, “Horizon [...] has not produced any documents to support the allocation of [those] expenses”. [Rosen Report, Figure 1 and ¶¶5.41-5.51.]

⁹⁵ On the issue of “other cost of sales”, which relate to the costs incurred by Horizon globally for expenses such as manufacturing operations, inventory, inventory adjustments, and freight and distribution for PROCYSBI in Canada, the point of disagreement accounts for [REDACTED] of the difference in our results on the net cash flows from PROCYSBI. In particular, our difference is based on the approach to allocating these costs. For my analysis, I allocated Horizon’s “other cost of sales” to PROCYSBI in Canada [REDACTED]

Mr.

69. The larger points of disagreement relate to the “Costs of Goods Sold” and “General and Administrative Expenses.”

(i) Growth Rate for Costs of Goods Sold

70. Cost of goods sold relates to the “production cost” of a good – in this case, the manufacturing, capsuling, and packaging of PROCYSBI units for sale in Canada.⁹⁶ For the purpose of my financial model, I used the actual per unit cost of goods sold by Horizon for PROCYSBI in Canada in 2017 and 2018, and Horizon’s cost of goods sold at standard cost for 2019. [REDACTED]

71. While Mr. Rosen and I agree on the basis for Horizon’s per-unit cost of goods sold, namely Horizon’s standard costs,⁹⁸ we disagree on the rate at which these costs would grow over time. Mr. Rosen’s cites to the Producer Price Index published by the U.S. Bureau of Labor Statistics (the “U.S. PPI”) and argues that the appropriate growth rate for the production costs of PROCYSBI in this case is [REDACTED]⁹⁹ This point of disagreement accounts for [REDACTED] of the difference between our results on the net cash flows from sale of PROCYSBI in Canada.

72. As a matter of economics, it is not appropriate to apply the U.S. PPI growth rate. First, the U.S. PPI tracks producer inflation in the U.S. However, as noted by Mr. Rosen, PROCYSBI is manufactured in Europe.¹⁰⁰ Moreover, this general producer inflation rate encompasses all

Rosen disagrees, stating that these costs should instead be allocated based on the number of “units sold by Horizon globally.” While an allocation based on units is a generally reasonable, it is inappropriate for the allocation of “other cost of sales”. As shown in Exhibit C to my September 2019 Report, [REDACTED]

[REDACTED] [Rosen Report, Figure 1 and ¶¶5.70-5.78.]

⁹⁶ Rosen Report, ¶¶5.31-5.35.

⁹⁷ Rosen Report, Figure 1, ¶¶5.28-5.29.

⁹⁸ Rosen Report, ¶5.38.

⁹⁹ Rosen Report, ¶¶5.32-5.35 and ¶-5.38; TOR0000001143; TOR0000001154.

¹⁰⁰ Rosen Report, Figure 9 and Figure 10.

industries in the U.S., including for example, electric power distribution, fruit and vegetable canning, industrial gas manufacturing, and grocery stores (among many others).¹⁰¹ None of these industries would reflect the cost pressures faced by pharmaceutical producers. Accordingly, the U.S. PPI growth rate would be completely inappropriate for PROCYSBI.

73. [REDACTED]

(ii) General and Administrative Expenses

74. General and Administrative expenses refer to costs for head office and business support functions (such as human resources, information technology, communications, business development, government and public affairs, finance, and corporate development and management) incurred by the Horizon Group for PROCYSBI in Canada.¹⁰³ [REDACTED]

75. [REDACTED]

¹⁰¹ U.S. Bureau of Labor Statistics, Producer Price Index Industry Data – Top Picks, available at <https://data.bls.gov/cgi-bin/surveymost?pc>.

¹⁰² Mr. Rosen cites to a single data point – [REDACTED] – to argue that [REDACTED] is not supported by Horizon’s data. However, based on my discussions with Horizon business representatives, [REDACTED] [Rosen Report, ¶5.35.]

¹⁰³ Rosen Report, ¶5.79, ¶5.84, and Figure 24.

76. Figure 5 below compares the line items I have included in my calculations to those included by Mr. Rosen

Figure 5: Horizon’s G&A Expense Line Items

Expense Item	Hay Report General & Administrative Expenses	Rosen Report General & Administrative Expenses
Human Resources	✓	✓
Finance	✓	✓
Legal	✓	✓
Information Technology	✓	✓
Business Development	✓	✓
Communications	✓	X
Government Affairs	✓	X
Public Affairs	✓	X
Patient Advocacy	✓	X
Corporate Development	✓	X
Corporate Management	✓	X
G&A Initiatives	✓	X
Facilities	✓	X
Corporate Security	✓	X

Source: Rosen Report, ¶5.87.

77. Mr. Rosen’s exclusion of Horizon’s communications, government and public affairs, patient advocacy, corporate development and management, G&A initiatives, facilities and security expenses makes no economic sense. Mr. Rosen appears to view his calculations of these other inputs as a “book-keeping” exercise – he excludes entire line items based on his assertion that Horizon has not provided detailed enough documentation. I am surprised that someone with Mr. Rosen’s experience would take this approach.

78. By disregarding these costs entirely, Mr. Rosen ignores the operating realities of a pharmaceutical company. Indeed, this approach suggests that, in Mr. Rosen’s view, Horizon (or any pharmaceutical company for that matter) can manage its global operations, including its operations in Canada, without incurring expenses for facilities, communications, public, government and patient relations, and corporate management, among others. These are necessary for the head office and business support functions provided by Horizon for PROCYSBI in Canada. Mr. Rosen himself notes that “Horizon only maintains a sales and

¹⁰⁴ Rosen Report, ¶¶5.87-5.88.

marketing team in Canada.” Accordingly, such expense items would have been incurred (and recorded) at the overall corporate level. As a matter of economics, these expenses should be included in the costs allocated to PROCYSBI in Canada.

V. UNDER MR. ROSEN’S ANALYSIS HORIZON COULD NOT RECOVER ITS COSTS

79. Mr. Rosen concludes that, based on his analysis, Horizon earns an IRR of [REDACTED] (at the 71% price reduction) and [REDACTED] (at the 80% price reduction) when viewed from the perspective of the Horizon Group.¹⁰⁵ At the 96% price reduction, [REDACTED]

[REDACTED]¹⁰⁶ As discussed below, even if one were to accept all of Mr. Rosen’s flawed calculations, the analysis shows that Horizon would [REDACTED]

[REDACTED] Moreover, simply correcting Mr. Rosen’s calculations only for his erroneous allocation of the PROCYSBI Commercialization Cost result in [REDACTED]

A. Mr. Rosen Fails to Account for the Cost of Capital

80. Mr. Rosen’s calculations appear to suggest that Horizon would earn a profit at the prices proposed under the Market Share Comparison Test and the Premium Comparison Test.

[REDACTED] Even if one were to assume that Mr. Rosen’s financial inputs were appropriate (which they are not), [REDACTED]

¹⁰⁵ Rosen Report, ¶1.23, Figure 4, ¶¶6.2-6.19 and Figure 27.

¹⁰⁶ At the price proposed under the Same Medicine Comparison Test, Mr. Rosen calculates that Horizon would earn – [REDACTED]. [Ibid.]

██████████¹⁰⁷ However, these amounts do not reflect Horizon's true costs because they do not incorporate Horizon's cost of capital.¹⁰⁸

81. It is important to put Mr. Rosen's calculation into context. ██████████
██████████
██████████
██████████.

82. What Mr. Rosen's calculations actually show is that Horizon would generate average *annual* cash flows of between ██████████
██████████ under Board Staff's Proposed Prices (see Figure 6 below). And again, these are undiscounted amounts, *i.e.*, they do not account for the time value of money or cost of risk. That is, these amounts do not fully reflect Horizon's costs because they do not incorporate its cost of capital.

83. As discussed in my September 2019 Report, the commercialization process for pharmaceutical products is a long and complex, characterized by large up-from investment costs, the returns from which will not be realized until many years in the future. Without discounting, equal weight is put on cash flows earned in ██████████ as those incurred today. All the while, Horizon would be facing a considerable cost of capital from financing the initial commercialization cost of PROCYSBI. Discounting reflects the time value of money – *i.e.*, the value of a dollar earned tomorrow is less than a dollar earned today. In the context of an investment in a new drug product, discounting reflects the cost of capital – that is, the cost of

¹⁰⁷ Rosen Report, ¶1.22, Figure 3, ¶¶6.1-6.11 and Figure 25.

¹⁰⁸ See Appendix B.2-3 for a detailed discussion of the concept of comparing the IRR to a company's cost of capital (specifically, its WACC).

The cost of capital measures the opportunity cost of obtaining financing (via debt or equity), based on the time value of money and the cost involved of the investment. As discussed in my September 2019 Report, the commercialization process for pharmaceutical products is a long and complex, characterized by large up-from investment costs, the returns from which will not be realized until many years in the future, if ever. The cost of capital compensates investors for bearing this risk. Indeed, several prominent studies on the economic cost to develop and commercialize new drug products have emphasized that the true cost of a new drug includes both the company's out-of-pocket expenses associated with the investment in R&D and the opportunity cost of capital incurred during the drug's development.

financing the investment upfront, while the returns from the investment will not be generated until well into the future.

B. Mr. Rosen’s IRR Analysis is Flawed

84. Mr. Rosen proceeds to calculate the IRR implied by these cash flow amounts, taking the IRR to “represent[t] the rate of return a project is expected to earn, which is generally used as a metric to assess the financial feasibility and viability of any project.”¹⁰⁹

85. However, Mr. Rosen’s analysis provides no information on whether Horizon can recover its costs under these two tests, and he never opines on whether these IRRs are adequate for a rare-disease drug like PROCYSBI.¹¹⁰ He offers no commentary on what these calculations mean, as a general comment or applied specifically to this case. He simply states his results, leaving readers to draw their own conclusions.¹¹¹ This is an important point because the IRR alone is meaningless: to determine whether a company will, in fact, earn a profit on investment, the IRR must be compared to the company’s cost of capital.¹¹²

86. The threshold rate of return that a company must earn for an investment to be profitable is referred to as the company’s Weighted Average Cost of Capital (“WACC”). WACC represents the weighted average of the cost required for each component of financing a commercial enterprise, generally comprising various forms of debt and equity, and can be objectively estimated at any point in time. Standard corporate finance techniques are available to estimate the WACC for a company, and such estimates are tabulated and published as a commercial service by various financial information providers.

¹⁰⁹ Rosen Report, ¶1.23 and ¶¶6.2-6.19.

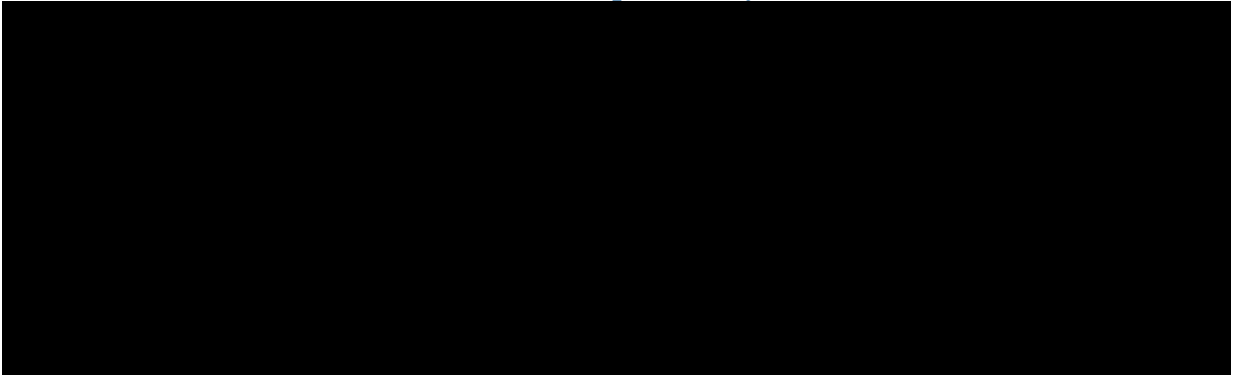
¹¹⁰ In my September 2019 Report and in my July 2020 Addendum Report, I focused on Horizon’s returns with respect to PROCYSBI in Canada under the Board’s Proposed Prices. In calculating these returns, I found that Horizon would [REDACTED]

¹¹¹ For example, at paragraph ¶6.22 of the Rosen Report, Mr. Rosen summarizes the calculations from his analysis, but offers no commentary as to what these calculations mean for this case. However, as acknowledged by, Prof. Schwindt – the economics expert retained by Board Staff – an appropriate market price must include “a normal return to entrepreneurial effort (*i.e.*, profits)”. [Schwindt Report, pp. 2-3.]

¹¹² I explain the concept of comparing the IRR to the company’s threshold weighted average cost of capital in Appendix B.

87. Based on my analysis, the relevant WACC rate for Horizon at the time of the Raptor acquisition is [REDACTED].¹¹³ Figure 6, below, compares the IRRs computed by Mr. Rosen to Horizon's WACC. As shown, Mr. Rosen's calculated IRRs are well below this rate, indicating that Horizon would be unable to recover its costs under at Board Staff's Proposed Prices.

**Figure 6: Horizon Return on Investment under Board Staff'
Per Rosen Report Analysis**



88. Specifically, this figure shows that even under Mr. Rosen's flawed analysis of Board Staff's Proposed Prices, [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

89. Thus, even if one were to assume that Mr. Rosen's financial inputs were appropriate (which they are not), this comparison demonstrates that Horizon would [REDACTED]

[REDACTED]

90. Moreover, simply correcting Mr. Rosen's calculations [REDACTED]

[REDACTED]

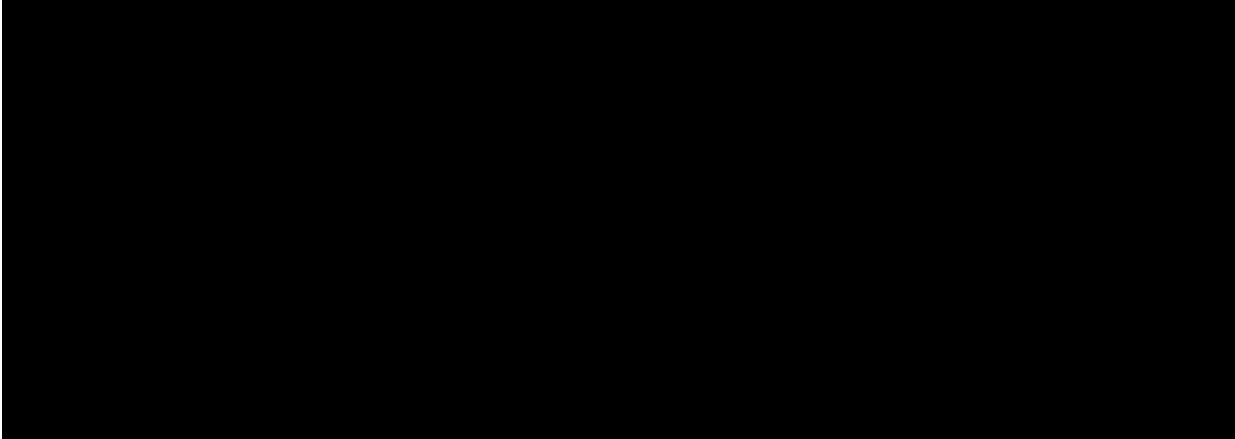
[REDACTED]

[REDACTED]

¹¹³ I provide the details of the relevant WACC rate for Horizon at the time of the Raptor acquisition in Appendix C.

[REDACTED]

**Figure 7: Horizon Return on Investment under Board Staff
Per Rosen Report Analysis, Correcting for Raptor Acquisition Cost Allocation**



91. I note that the conclusion that Horizon will earn a [REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]
[REDACTED]

92. In summary, all of the analyses undertaken in this case – as well as Horizon’s ordinary course analysis – show that Horizon would [REDACTED]
[REDACTED]

¹¹⁴ See Annex 1 and Annex 2 in Appendix D for details of my calculations.

¹¹⁵ TOR0000001046.

¹¹⁶ Horizon Pharma Annual Report for 2018, p. 95.

C. Horizon's Losses Under Board Staff's Proposed Prices are Understated

93. It is important to note that the financial models Mr. Rosen and I use may understate the [REDACTED] under Board Staff's Proposed Prices. This is because the forecasted revenues we both use (recall, Mr. Rosen has adopted my sales volumes and his analysis and calculations) are potentially inflated.

(i) Board Staff Propose Even Larger Price Reductions

94. Board Staff's proposed price reductions are higher than those used in mine and Mr. Rosen's analysis. Board Staff are seeking a reduction in the ex-factory price of PROCYSBI of between 96% and 98% under the Same Medicine Comparison Test, 80% and 92% under the Market Share Comparison Test, and 71% and 73% under the Premium Comparison Test. To be conservative, I used the lower bound of each price range (*i.e.*, 96%, 80% and 71%). I did not need to consider the upper bound of each price range because, even at the lower range, [REDACTED] However, Mr. Rosen adopted these same figures without considering the range of the proposed price reductions. Had we used the higher range of Board Staff's proposed reductions, [REDACTED]

(ii) Loss of Exclusivity

95. For the purpose of forecasting the sales volumes of PROCYSBI in Canada, I was instructed to assume that Horizon would have market exclusivity in Canada through to the expiry of PROCYSBI's patents in [REDACTED].¹¹⁷ This assumption resulted in Horizon's sales units increasing throughout the term. However, if Horizon were to lose market exclusivity (for example, as a result of entry by a generic manufacturer) during this period, its sales volume would be expected to decrease substantially. In my experience, generic entry can erode the sales volume of the branded reference by as much as 50% within a year or two.¹¹⁸ If Horizon

¹¹⁷ See, *e.g.*, Figure 10 below.

¹¹⁸ See *e.g.*, Grabowski, H. G., & Vernon, J. M. (1992). Brand loyalty, entry, and price competition in pharmaceuticals after the 1984 Drug Act. *The Journal of Law and Economics*, 35(2), 331-350; Grabowski, H., & Vernon, J. (1996). Longer patents for increased generic competition in the US. *PharmacoEconomics*, 10(2), 110-123.

As noted by the Competition Bureau in its study of the generic drug sector in Canada, "[o]nce generic versions of brand-name products are placed on provincial formularies and are designated

were to lose its market exclusivity during the period of study, that would likely result in a decrease in PROCYSBI sales units in Canada, and in turn, result in Horizon incurring even larger loss on investment than calculated above.

D. A Negative Return on Investment is Inconsistent with the PMPRB's New Guidelines

96. As discussed above, even if one were to assume that Mr. Rosen's financial inputs were appropriate (which they are not), once the cost of capital is considered, it becomes clear that Horizon would [REDACTED]

[REDACTED] This is inconsistent with the PMPRB's New Guidelines, which were published on October 23, 2020 and take effect in January 2021.¹¹⁹

97. The principle that producers of drugs for rare diseases must have a fair opportunity to earn a reasonable return (via a relatively higher price) in order to incentivize development is reflected in the PMPRB's New Guidelines. For example, in the background section for the consultation stage of the New Guidelines, the PMPRB states that:¹²⁰

Increasing awareness and technological advances means that more Canadians are being diagnosed with and seeking treatment for rare diseases and disorders. Consistent with the Government of Canada's commitment in Budget 2019 to support Canadians with rare diseases, the Draft Guidelines include a MRP adjustment for patented medicines for rare diseases and disorders with small patient population (*i.e.*, small market size due to low prevalence). The adjustment effectively means that ceiling prices for the Net Price of patented medicines for rare diseases and disorders realizing small volumes of sales will be higher than those for more common conditions.

98. I have been advised that, under the New Guidelines, drugs like PROCYSBI that are classified as Category I would have their Maximum Rebated Price ("MRP") ceiling for their

as interchangeable, they essentially become commodity products." ["Canadian Generic Drug Sector Study", available at [https://www.competitionbureau.gc.ca/eic/site/cb-bc.nsf/vwapj/Competition%20Bureau%20Generic%20Drug%20Sector%20Study.pdf/\\$FILE/Competition%20Bureau%20Generic%20Drug%20Sector%20Study.pdf](https://www.competitionbureau.gc.ca/eic/site/cb-bc.nsf/vwapj/Competition%20Bureau%20Generic%20Drug%20Sector%20Study.pdf/$FILE/Competition%20Bureau%20Generic%20Drug%20Sector%20Study.pdf)]

¹¹⁹ PMPRB Guidelines, Effective January 1, 2020, available at <https://www.canada.ca/content/dam/pmprb-cepmb/documents/legislation/guidelines/PMPRB-Guidelines-en.pdf> ("PMPRB New Guidelines").

¹²⁰ PMPRB Draft Guidelines Consultation, available at <https://www.canada.ca/en/patented-medicine-prices-review/services/consultations/draft-guidelines.html>.

first \$12 million of annual revenues set to their Maximum List Price (“MLP”). As shown in Figure 6, below, for “Grandfathered patented medicines” (*i.e.*, patented medicines for which a DIN was assigned prior to August 2019) this would be the lower of (i) the highest international price in the PMPRB11 countries, or (ii) the ceiling under the Guidelines applicable prior to the New Guidelines.¹²¹ For PROCYSBI, I understand that this MLP would essentially be the current Median International Price.¹²²

Figure 8: MRP under the Draft Guidelines

Annual sales ¹	MRP	Incremental MLP adjustment factor	MLP adjustment factor
< \$12M	MLP		0%
\$12M-\$50M	Greater of PEP and Cap	0%	$\frac{\$12M - \$12M \cdot (MRP/MLP)}{\text{Sales}} + (MRP/MLP)$
\$50M-\$100M		-25%	$\frac{\$21.5M - \$9M \cdot (MRP/MLP)}{\text{Sales}} + (1 - 25\%) \cdot (MRP/MLP)$
> \$100M		-35%	$\frac{\$32M - \$7.8M \cdot (MRP/MLP)}{\text{Sales}} + (1 - 35\%) \cdot (MRP/MLP)$

Source: PMPRB New Guidelines, p. 36.

99. I have also been advised that, for revenues above this \$12 million cap, rebates will be due at a rate dependent on the drug’s therapeutic classification. As shown in Figure 9, below, the potential rebates due would be no more than 50% of the MLP.

¹²¹ PMPRB New Guidelines, ¶33 and ¶71.

¹²² I understand that the new PMRRB11 countries do not include the U.S. Nevertheless, as shown in Figure 3 from my September 2019 Report, an excerpt of which is provided below for ease of reference, Horizon’s current ex-factor price for PROCYSBI in Canada would remain below the median international price excluding the U.S.

MIPC Excluding the U.S.

Country	Price in CAD\$ as at Apr 2017	
	25mg	75mg
Canada	\$0.4140	\$0.4140
Other Countries in which PROCYSBI is Sold		
Germany	\$0.4179	\$0.4179
United Kingdom	\$0.4115	\$0.4115
United States	\$4.2136	\$1.4045
Median	\$0.4179	\$0.4179
Median Excluding the US	\$0.4147	\$0.4147

Figure 9: Price Adjustments under the Draft Guidelines

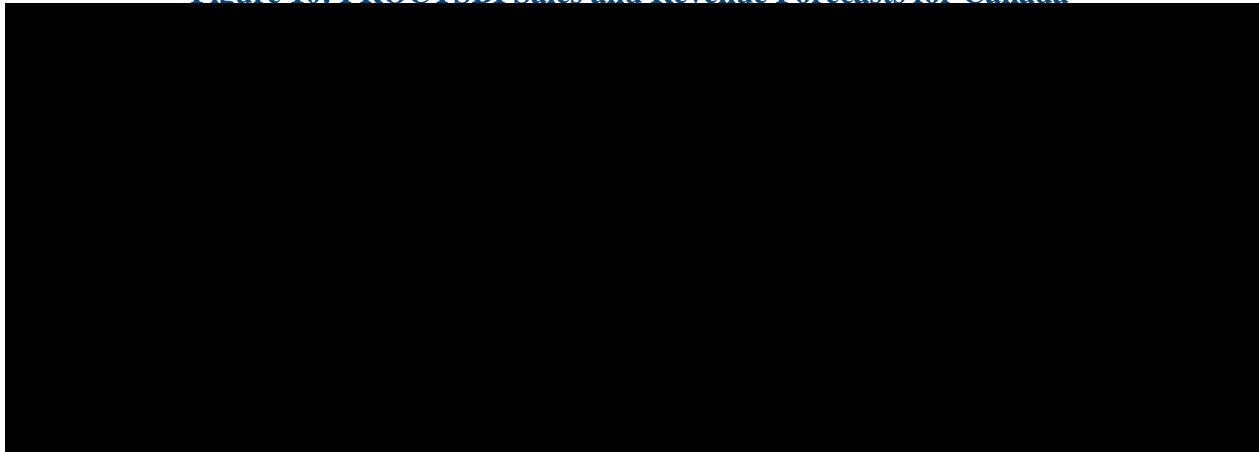
Therapeutic Criteria Level (See Appendix E - The Scientific Review Process)	PVT	Reduction Cap off MLP
Level I	\$200K/QALY	20%
Level II	\$150K/QALY	30%
Level III	\$150K/QALY	40%
Level IV	\$100K/QALY	50%
Pharmacoeconomic analysis does not report an ICUR	Median of dTCC subject to 50% cap	
No pharmacoeconomic analysis filed	50% of MLP	

Source: PMPRB New Guidelines, p. 35.

100. For PROCYSBI, this means that any potential rebates would be relatively minimal compared to what is being requested by Board Staff. As shown in Figure 10, below, both mine and Mr. Rosen’s forecasts for PROCYSBI revenues in Canada [REDACTED]

[REDACTED]¹²³ Accordingly, I understand that any rebates that would be payable under the New Guidelines would result in a (net) price to Horizon that is far higher than Board Staff’s Proposed prices.

Figure 10: PROCYSBI Sales and Revenue Forecasts for Canada



101. For example, even under a rebate rate of 50% of the MLP, if Horizon were to make revenues of [REDACTED] (*i.e.*, after PMPRB rebates) would equate to a price reduction [REDACTED] from Horizon’s current ex-factory price.¹²⁴ In contrast, Board Staff’s Proposed Prices involve price reductions of between 71% and 96%.

¹²³ Note, in Figure 10, 2034 is only a half year, through to June.

¹²⁴ \$12 million at its MLP and the other \$12 million at 50% of its MLP, *i.e.*, an average net price of 75% of its MLP.


In short, the price reductions sought by Board Staff are inconsistent with the New Guidelines, as they have been explained to me.

VI. CONCLUSION

102. As demonstrated above, even under Mr. Rosen's flawed methodology, Horizon would [REDACTED] [REDACTED] Indeed, once Mr. Rosen's analysis is corrected, it becomes clear that Horizon would [REDACTED] [REDACTED] Accordingly, Board Staff's Proposed Prices are inconsistent with the economic principle that the price of a new drug product should provide the manufacturer with a reasonable opportunity to recover the costs associated with developing and commercializing the new drug – a principle which the economics expert retained by Board Staff – Prof. Schwindt – endorses.

103. Thus, regulatory restrictions that prevent manufacturers from recovering upfront costs for new rare disease drugs can be expected to undermine companies' incentives to invest in the development of rare disease drugs that benefit Canadian patients.

Signed November 3rd, 2020



Joel W. Hay

Appendix A: Scope of Review

In preparing this report, in addition to the information and documents relied on for my previous September 2019 Report, July 2020 Addendum and October 2020 Responding Report in this matter, I have reviewed and relied upon the information from documents and listed below:

A. Expert Reports

- i. Expert Report of Howard Rosen (Secretariat), dated October 6, 2020.

B. Horizon Productions

- i. TOR0000001046.
- ii. TOR0000001143.
- iii. TOR0000001154.

C. Publicly Available Information

- i. Bank of Canada, Canadian Bond Yields: 10-Year Lookup, <https://www.bankofcanada.ca/rates/interest-rates/lookup-bond-yields>.
- ii. Brealey, R., Myers, S., and Franklin, A. (2020). Chapter 5: Net Present Value and Other Investment Criteria, in *Principles of Corporate Finance, 13th Edition*. New York: McGraw-Hill.
- iii. Brealey, R., Myers, S., and Franklin, A. (2020). Chapter 8: Portfolio Theory and the Capital Asset Pricing Model, in *Principles of Corporate Finance, 13th Edition*. New York: McGraw-Hill.
- iv. Competition Bureau, “Canadian Generic Drug Sector Study”, available at [https://www.competitionbureau.gc.ca/eic/site/cb-bc.nsf/vwapj/Competition%20Bureau%20Generic%20Drug%20Sector%20Study.pdf/\\$FILE/Competition%20Bureau%20Generic%20Drug%20Sector%20Study.pdf](https://www.competitionbureau.gc.ca/eic/site/cb-bc.nsf/vwapj/Competition%20Bureau%20Generic%20Drug%20Sector%20Study.pdf/$FILE/Competition%20Bureau%20Generic%20Drug%20Sector%20Study.pdf).
- v. Damodaran, A., “The Value of Intangibles”, available at <http://people.stern.nyu.edu/adamodar/pdfiles/ovhds/dam2ed/intangibles.pdf>.
- vi. Dimson, E., Marsh, P. and Staunton, M. (2016). *The Credit Suisse Global Investment Returns Yearbook 2016*, available at <https://www.credit->

suisse.com/media/assets/corporate/docs/about-us/research/publications/csri-returns-yearbook-2016.pdf.

- vii. Elmonem, MA. et al. (2016). Cystinosis: A Review. *Orphanet Journal of Rare Diseases*, 11(47), 1-17.
- viii. Federal Reserve Economic Data (FRED St. Louis), 30-Year Treasury data (trailing 5-Day average of series DGS30 as of 10/26/2020), available at <https://fred.stlouisfed.org/series/DGS30>.]
- ix. Fernandez, P., Ortiz, A., and Acin, I F. (2017). Market Risk Premium used in 71 countries in 2016: a survey with 6,932 answers. *Journal of International Business Research and Marketing*, 2(6), 23-31.
- x. Grabowski, H. G., & Vernon, J. M. (1992). Brand loyalty, entry, and price competition in pharmaceuticals after the 1984 Drug Act. *The Journal of Law and Economics*, 35(2), 331-350.
- xi. Grabowski, H., & Vernon, J. (1996). Longer patents for increased generic competition in the US. *PharmacoEconomics*, 10(2), 110-123.
- xii. Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2016.
- xiii. Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2017.
- xiv. Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2018.
- xv. Horizon Pharma Public Limited Company Form 10-K for the fiscal year ended December 31, 2019.
- xvi. KPMG Corporate Tax Rates, “Federal and Provincial/Territorial Tax Rates for Income Earned by a General Corporation Effective January 1, 2016 and 2017”, available at <https://home.kpmg/content/dam/kpmg/ca/pdf/2016/10/income-tax-rates-for-general-corporations-2016-and-2017.pdf>.
- xvii. KRYSTEXXA website, available at <https://www.krystexxa.com>.
- xviii. Market-Risk-Premia.com, Market Risk Premia – Canada, available at <http://www.market-risk-premia.com/ca.html>.
- xix. McFetridge, D.G., “The Definition of Making and Marketing Costs for Purposes of Section 85(2) of The Patent Act”, Patented Medicines Prices

- Review Board, available at <http://www.pmprb-cepmb.gc.ca/view.asp?ccid=1071&wbdisable=true>.
- xx. PENNSAID website, available at <https://www.pennsaid.com>.
- xxi. Pertile, P. et al. (2018). Free-Riding in Pharmaceutical Price Regulation: Theory and Evidence. *Mimeo*, Department of Economics, University of York. No. 18/04.
- xxii. PMPRB Draft Guidelines Consultation, available at <https://www.canada.ca/en/patented-medicine-prices-review/services/consultations/draft-guidelines.html>.
- xxiii. PMPRB Guidelines, Effective January 1, 2020, available at <https://www.canada.ca/content/dam/pmprb-cepmb/documents/legislation/guidelines/PMPRB-Guidelines-en.pdf>.
- xxiv. PROCYSBI, Health Canada NOC Database, available at <https://health-products.canada.ca/noc-ac/info.do?lang=en&no=19408>.
- xxv. QUINSAIR Product Monograph, available at <https://www.hzndocs.com/QUINSAIR-Product-Monograph-English.pdf>.
- xxvi. RAYOS website, available at <https://www.rayosrx.com>.
- xxvii. Safety/Effectiveness Study of Cysteamine Bitartrate Delayed-release Capsules (RP103) in Cysteamine Treatment Naive Patients with Cystinosis, available at <https://clinicaltrials.gov/ct2/show/NCT01744782>.
- xxviii. Statista, Average market risk premium in Canada 2011-2020, available at <https://www.statista.com/statistics/664845/average-market-risk-premium-canada>.
- xxix. U.S. Bureau of Labor Statistics, Producer Price Index Industry Data – Top Picks, available at <https://data.bls.gov/cgi-bin/surveymost?pc>.
- xxx. U.S. FDA Approval Letter, DUEXIS NDA, available at https://www.access.data.fda.gov/drugsatfda_docs/appletter/2011/022519s000ltr.pdf.
- xxxi. U.S. FDA Approval Letter, PENNSAID NDA, available at https://www.access.data.fda.gov/drugsatfda_docs/appletter/2009/020947s000ltr.pdf.

Appendix B: Overview of Return on Investment Analysis

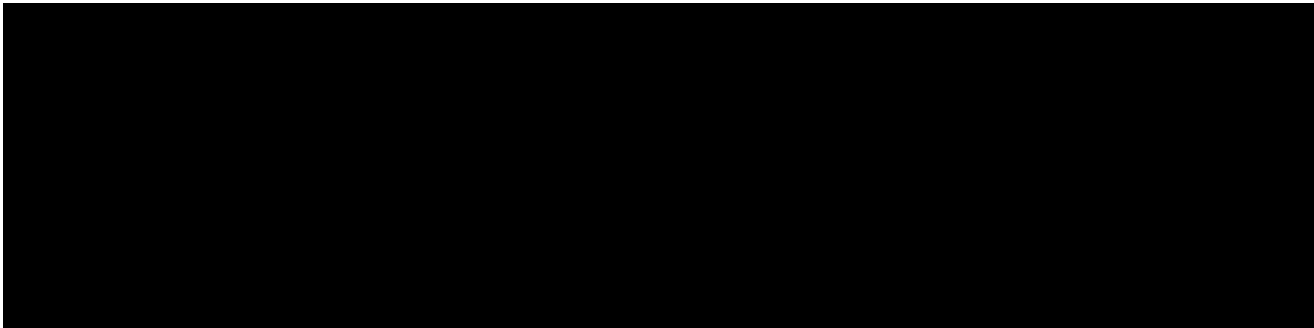
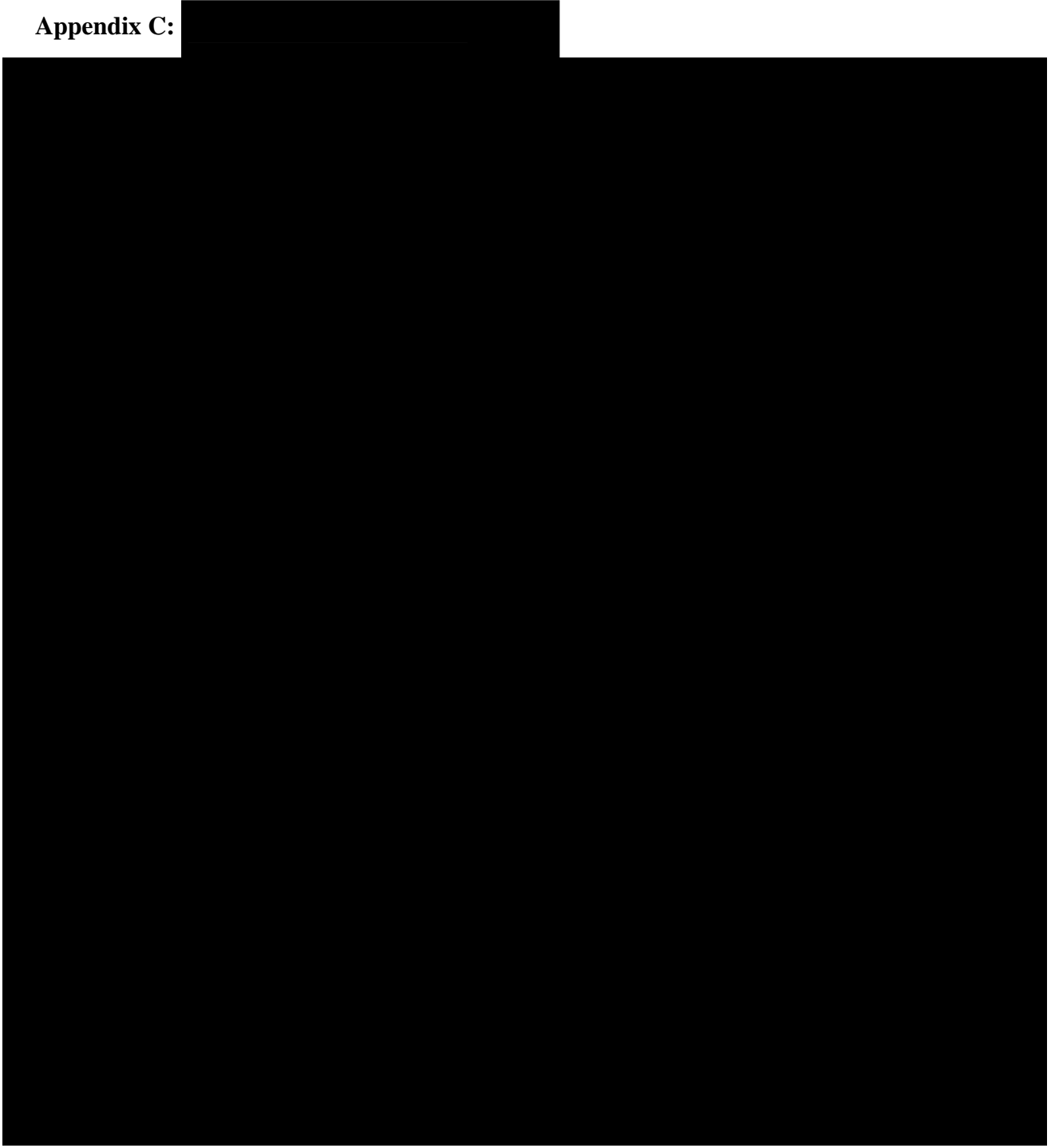
104. The Internal Rate of Return (“IRR”) of an investment is simply the average annual rate of return (or more precisely, the yield) that is expected to be realized by the investment over its lifetime, controlling for the timing of those cash flows.¹²⁵ In doing so, the IRR recognizes that cash flows earned today are more valuable than those earned tomorrow (i.e., “time value of money”). This is because if one were to place a dollar received today in the bank, one could withdraw the original dollar along with a year’s worth of interest on that dollar one year from today. Because of the time value of money, returns earned on an investment early on are more valuable than returns that are earned later, even in the absence of inflation.

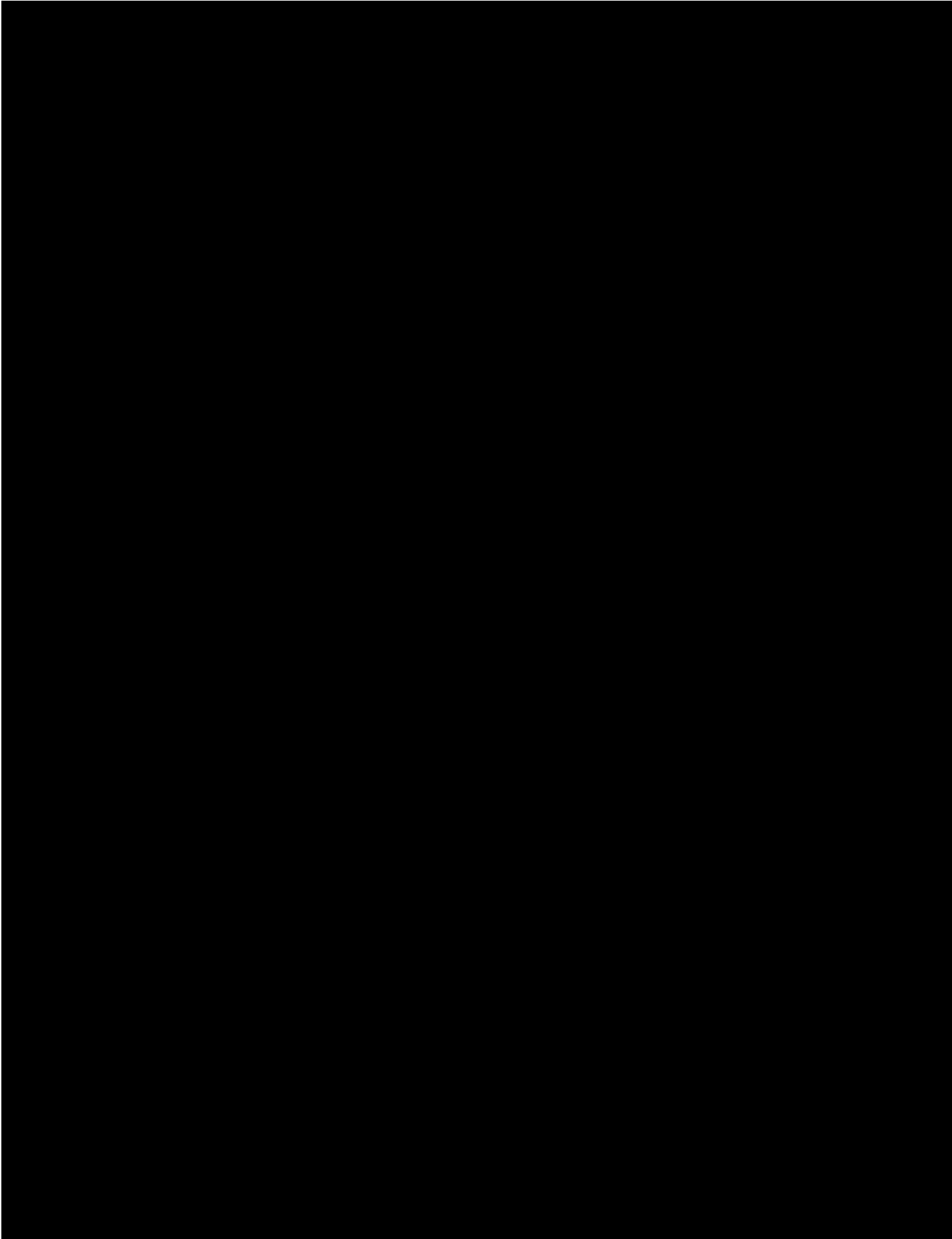
105. From an economic standpoint, if the IRR from a company’s product is in line with what the company could have earned from an alternative investment with similar risks, the return on investment from the product is not excessive and therefore the prices being charged are also not excessive. Conversely, if the IRR from a company’s product is below what the company could have earned from an alternative investment, the return from the product is inadequate and therefore the prices being proposed are, as a matter of economics, unjustified. The benchmark against which one tests IRR for economic profitability is called the *hurdle rate*, and the minimum hurdle rate that makes an investment’s return adequate is obtained from the *weighted average cost of capital* (“WACC”) of the investment. The WACC relevant to the PROCYSBI cash flows is estimated and discussed further in Appendix C.

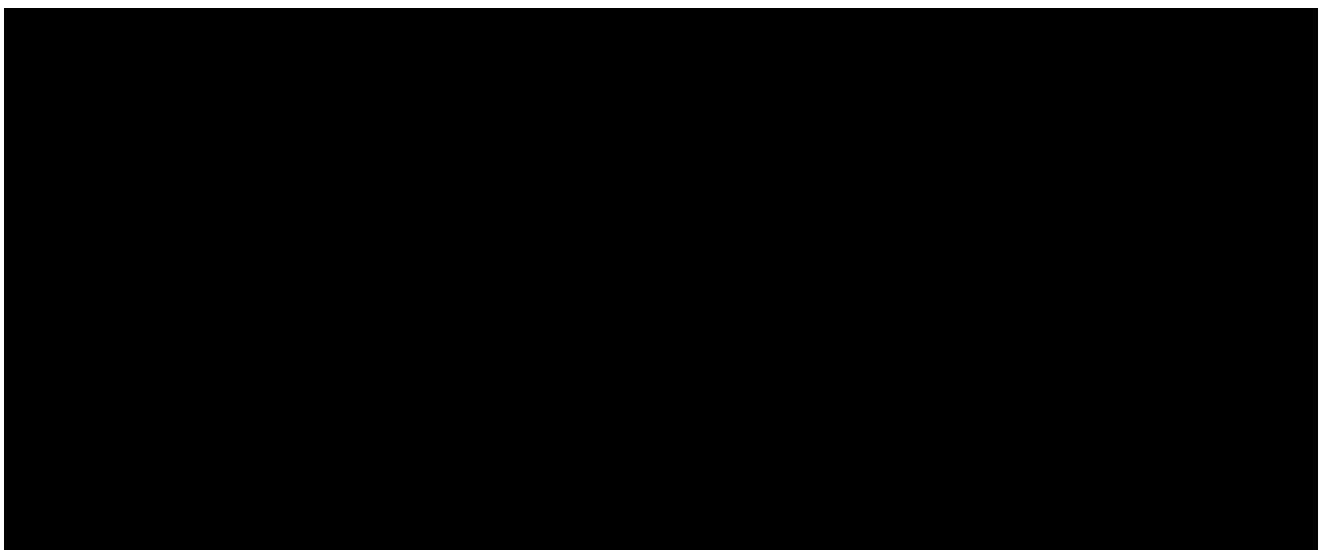
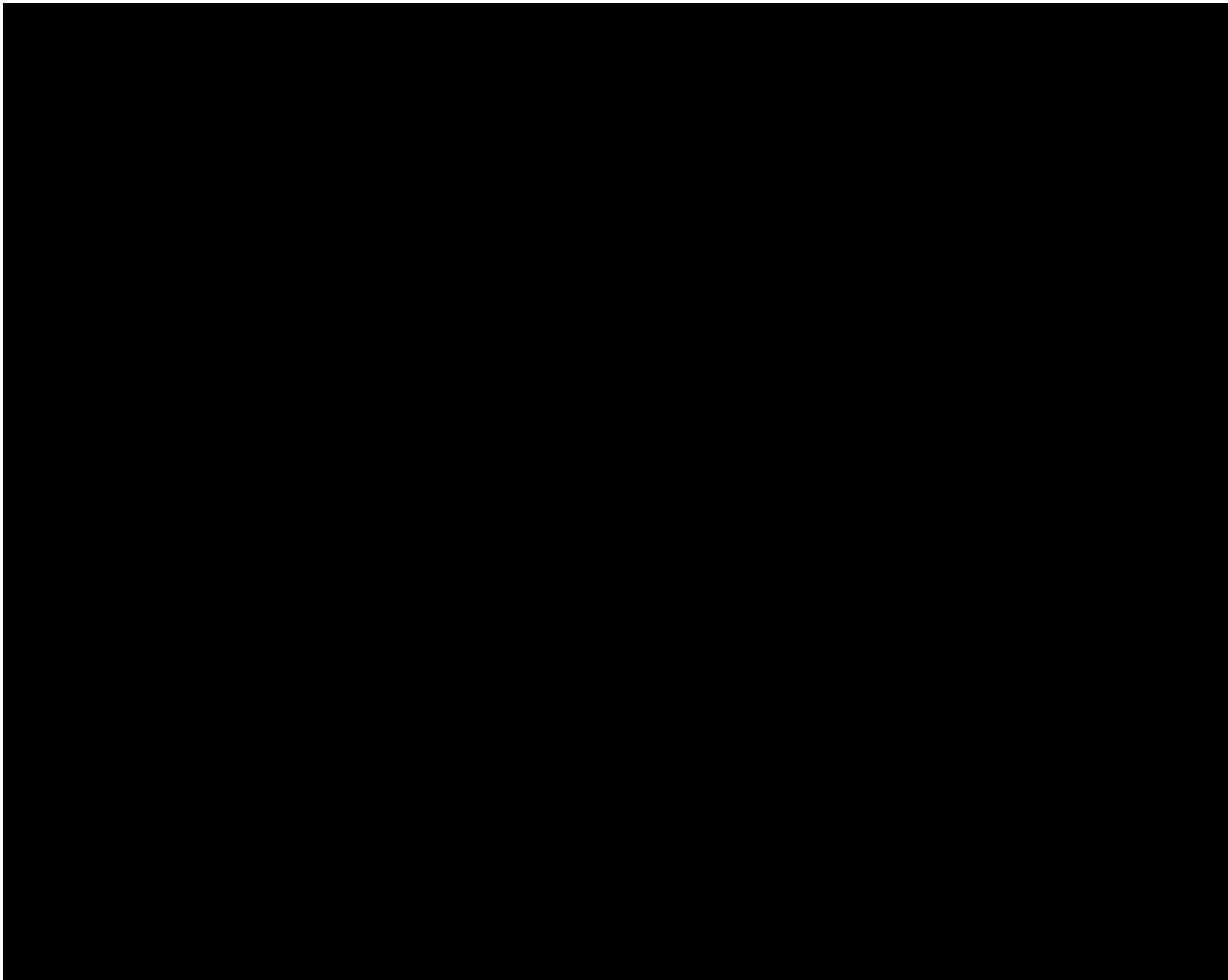
¹²⁵ The IRR can also be seen as the discount rate that sets the present value of future cash flows equal to the cost of the initial investment.

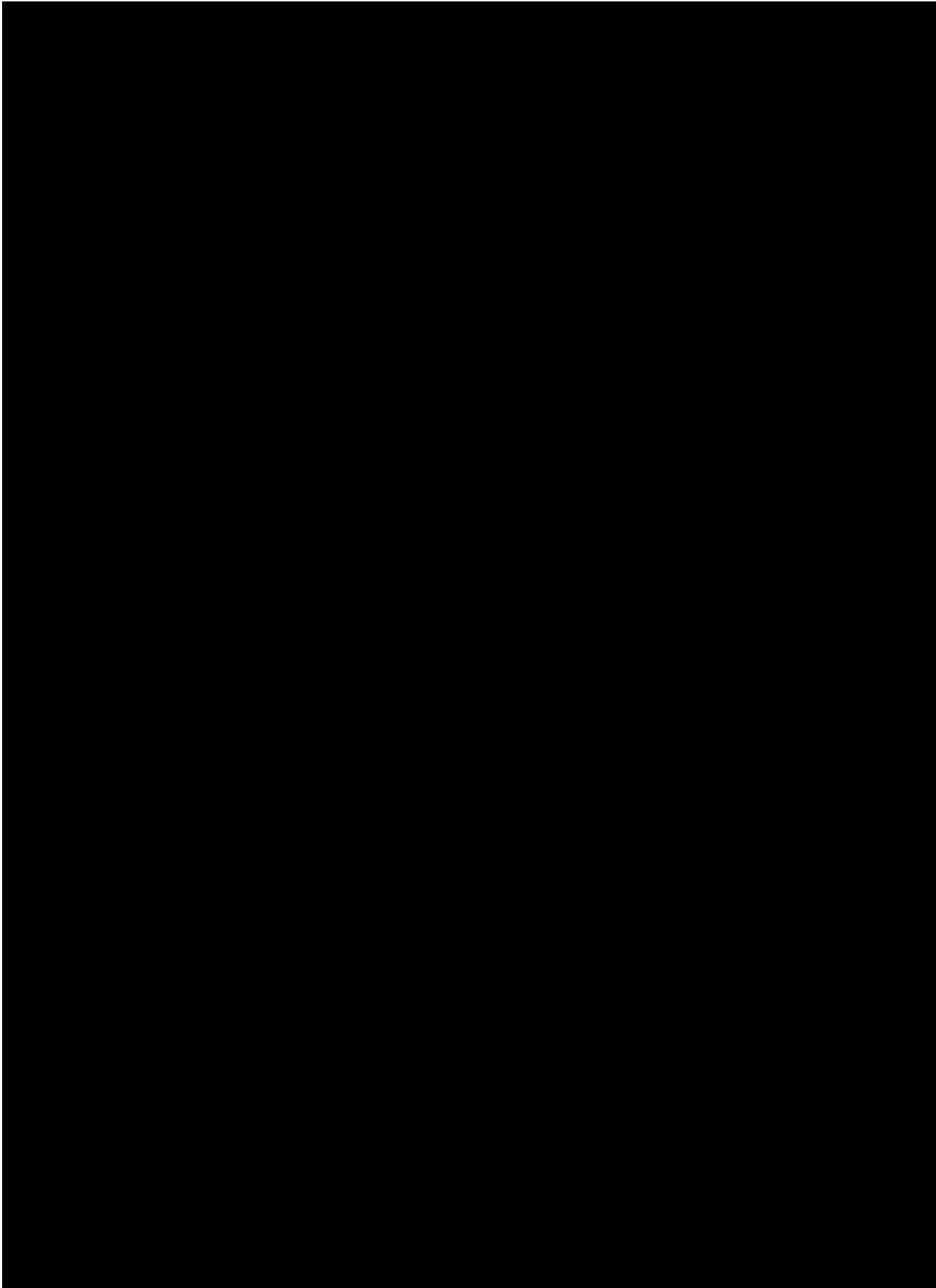
For a general discussion of IRR see, e.g., Brealey, R., Myers, S., and Franklin, A. (2020). Chapter 5: Net Present Value and Other Investment Criteria, pp. 114-116, in *Principles of Corporate Finance, 13th Edition*. New York: McGraw-Hill.

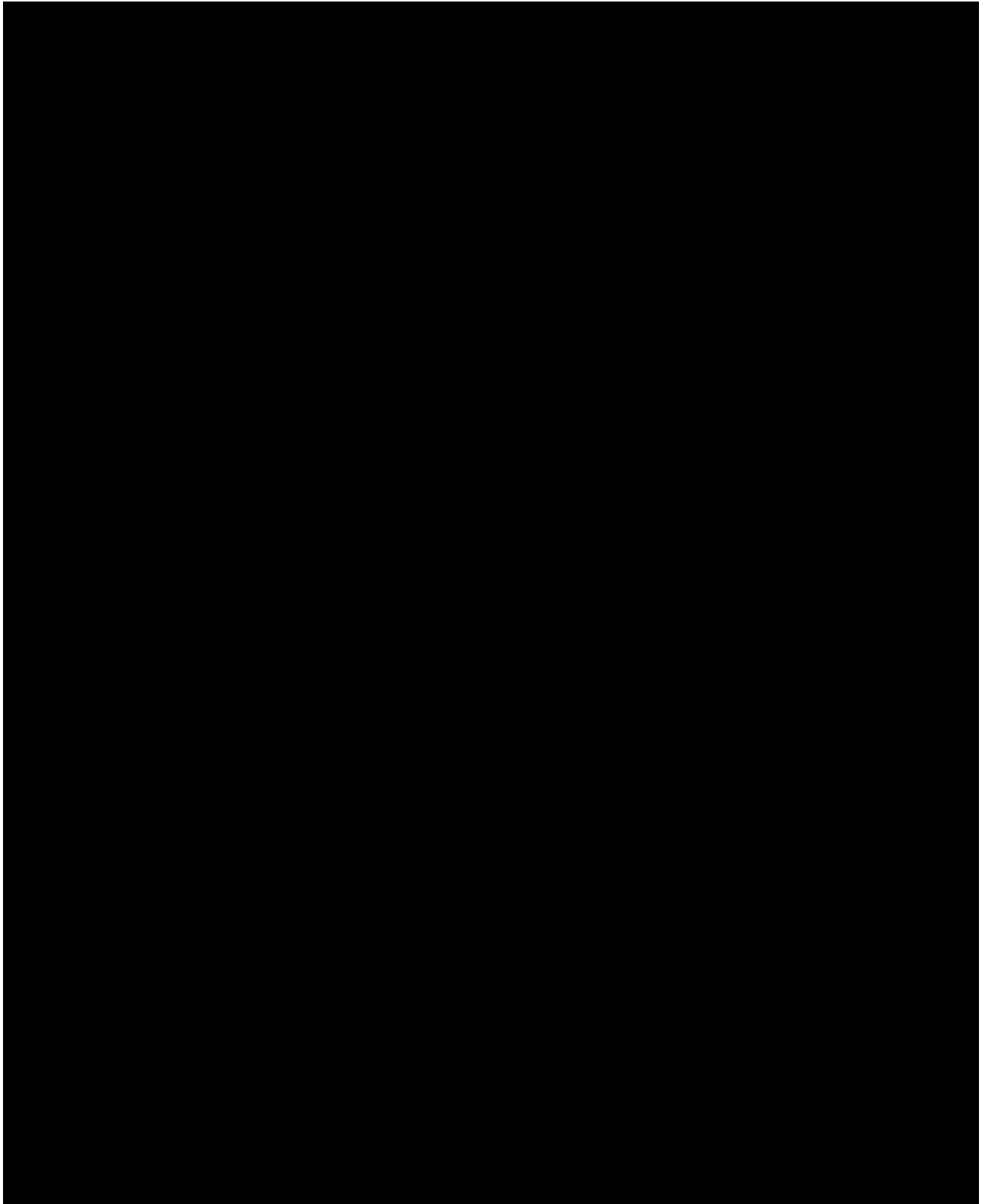
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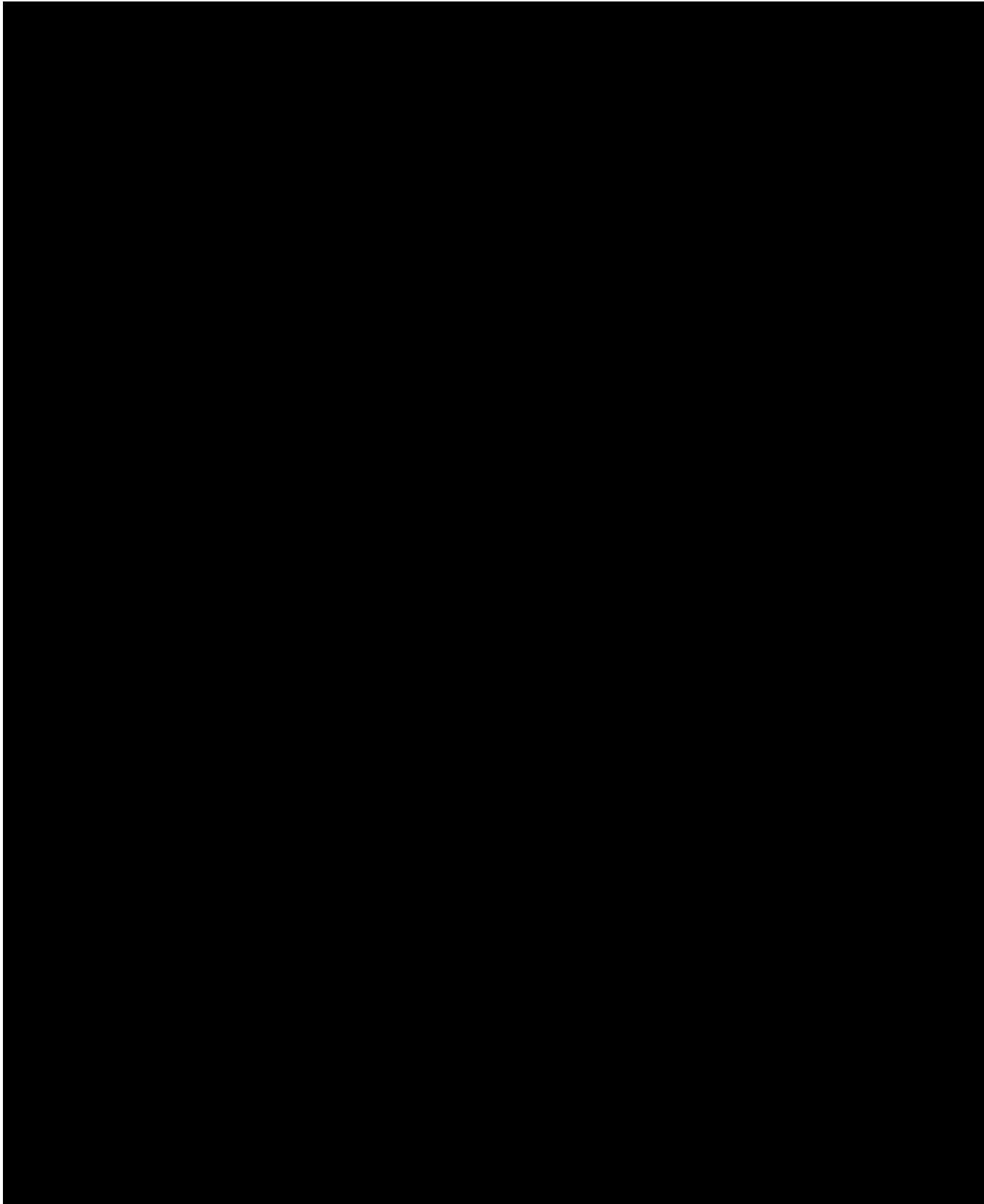












Appendix D: Schedules to Re-Analysis of Rosen Report Calculations

