

THE CURRENT AND FUTURE COST OF ORPHAN DRUGS IN CANADA

A PUBLIC PAYER BUDGET IMPACT ANALYSIS

Forte L, Malmberg C, Mann K, Jupe K, Chow G, Lech R, Kulik R

INTRODUCTION

According to Health Canada, rare diseases are life-threatening, debilitating or serious and chronic conditions that affect a small number of individuals [1]. Similar to the European Medicines Agency (EMA), Health Canada has adopted a definition of a rare disease as one that affects fewer than 50 persons in a population of 100,000 [1,2]. Canadian policy makers have expressed concern over the cost and potential growth in public expenditures on orphan drugs for rare diseases (DRDs) [1,3,4].

OBJECTIVES

The primary objective of the study was to quantify the current and anticipated total future public expenditures on DRDs in Canada. Secondary objectives were to provide context for the results by comparing current DRD spending to: 1) the top 20 public drug expenditures; 2) total public drug expenditures; and 3) other public and consumer expenditures.

METHODOLOGY

Identification of DRDs

To ensure that all currently funded DRDs and those that could potentially come to market in Canada were identified, a comprehensive search was performed. Currently available drugs were screened from the EMA Community Register of Orphan Medicinal Products [5] and two peer reviewed Canadian publications [6,7]. Future pipeline drugs were screened from the Pharmaceutical Research and Manufacturers of America (PhRMA) Medicines in Development for Rare Disease report [8]. Figure 1 shows the inclusion and exclusion criteria. Oncology drugs were excluded because rarity does not drive drug prices in oncology [9].

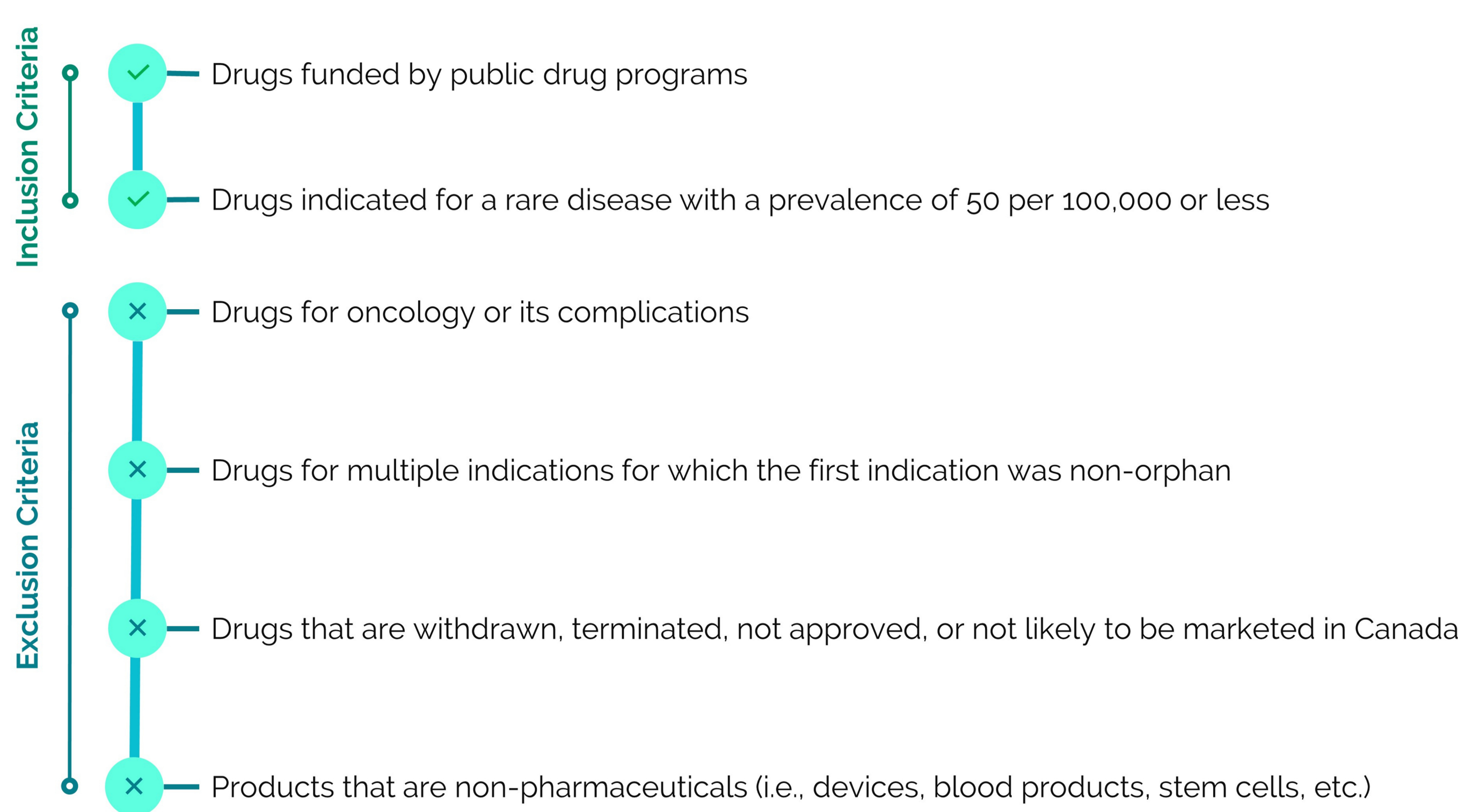


Figure 1. Inclusion and exclusion criteria used for screening DRDs

Quantification of DRD Expenditures

Historical expenditures (2010 to 2018) for DRDs currently funded by Canadian public drug programs were obtained from a claims database [10]. Freedom of Information (FOI) requests were sent to each public drug program to obtain reimbursement data for drugs funded through dedicated DRD programs, which are not included in claims data. All historical costs were extrapolated in a linear manner to 2025.

Future expenditures for pipeline DRDs expected to reach the Canadian market were also projected to 2025. Extrapolations were based on development phase, regulatory success rate, expected year of public funding, prevalence, and expected weighted average annual cost. Additional assumptions are presented in Figure 2.

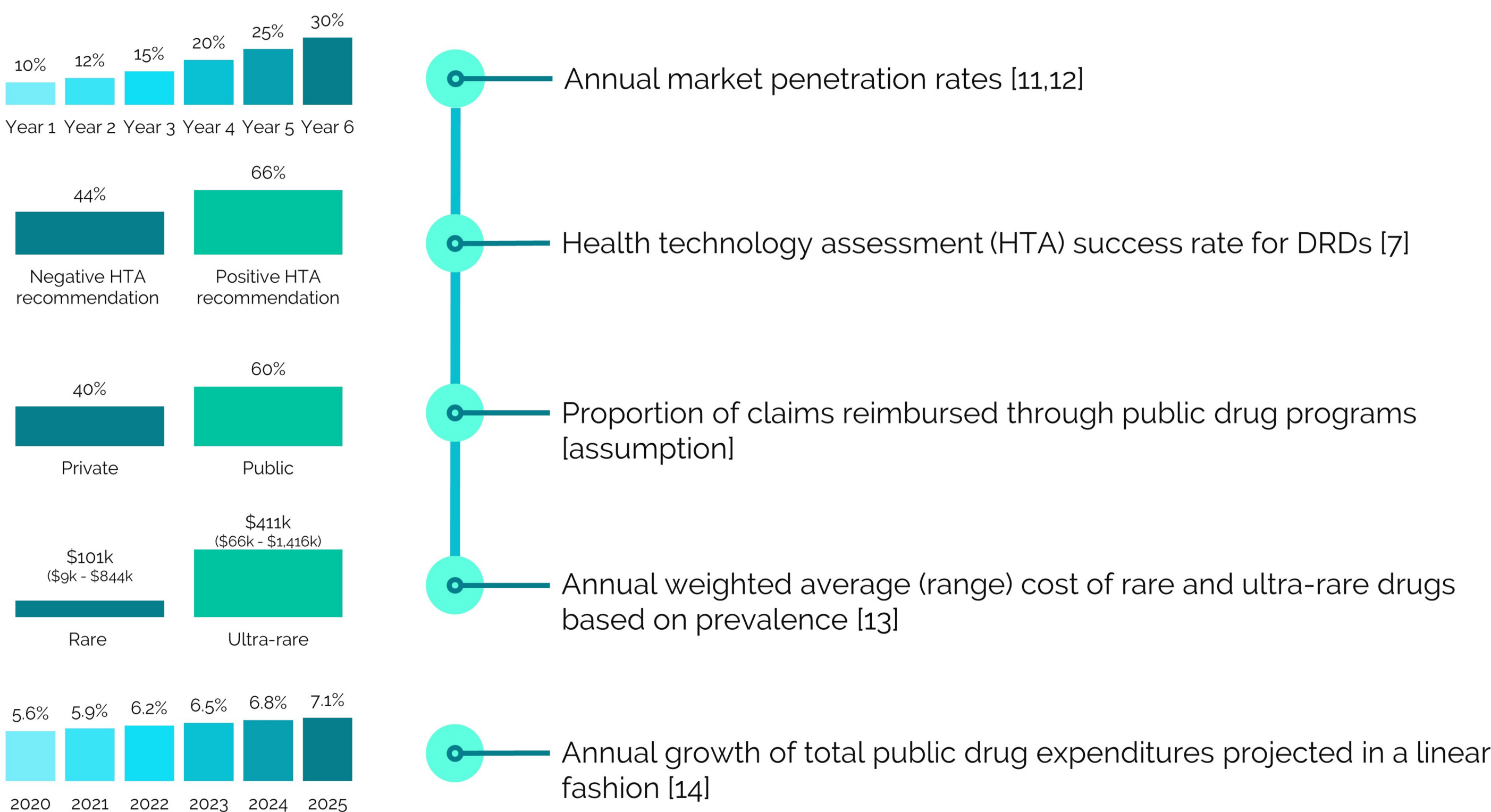


Figure 2. Assumptions used to calculate the public expenditures for the anticipated DRDs in the pipeline

The future public expenditures for currently funded and pipeline drugs were totaled for each year to 2025. The total projected DRD costs were then compared with: 1) the total costs for the drugs with the 20 highest Canadian public expenditures (obtained from the historical claims data); and 2) the total public expenditures for all drugs (sourced from the Canadian Institute for Health Information (CIHI); 2014 to 2018) projected in a linear manner to 2025 [14].

The base case and sensitivity analyses (SA) were defined as:

1. Base case: Extrapolation of historical DRD costs + projection of DRDs with EMA or FDA approval;
2. SA 1: Extrapolation of only historical DRD costs;
3. SA 2: Inclusion of DRDs in Phase 3 development as part of the pipeline;
4. SA 3: SA 2 + assumption of a higher HTA success rate (90%);
5. SA 4: Assumptions of a 35% confidential price reduction through manufacturer risk-sharing mechanisms (i.e., rebates, caps, outcome-based agreements, etc.) on the base case, SA 1, SA 2, and SA 3.

RESULTS

After removing duplicates, a total of 246 unique drugs were identified from the EMA list and the two Canadian publications. After screening for eligibility, 44 were determined to be DRDs approved by Health Canada. Public expenditures were available for 32 DRDs from claims data that was supplemented with FOI requests. Data for the remaining 12 DRDs were considered in the pipeline analysis because they were not yet funded. After screening, 60 additional pipeline drugs not yet approved in Canada were identified from the PhRMA list.

The total public drug program expenditure for currently funded DRDs grew from \$15 million in 2010 (11 drugs) to \$79 million in 2014 (18 drugs) and \$235 million in 2018 (32 drugs). From 2014 to 2018, total public drug expenditures in Canada increased from \$11.4 to \$14.4 billion. Expenditures for the funded DRDs as a proportion of total public drug expenditure increased from 0.7% in 2014 to 1.6% in 2018. The future public expenditures (extrapolated + pipeline) are anticipated to be \$280 million in 2019, growing to \$1,394 million in 2025 (Figure 3).

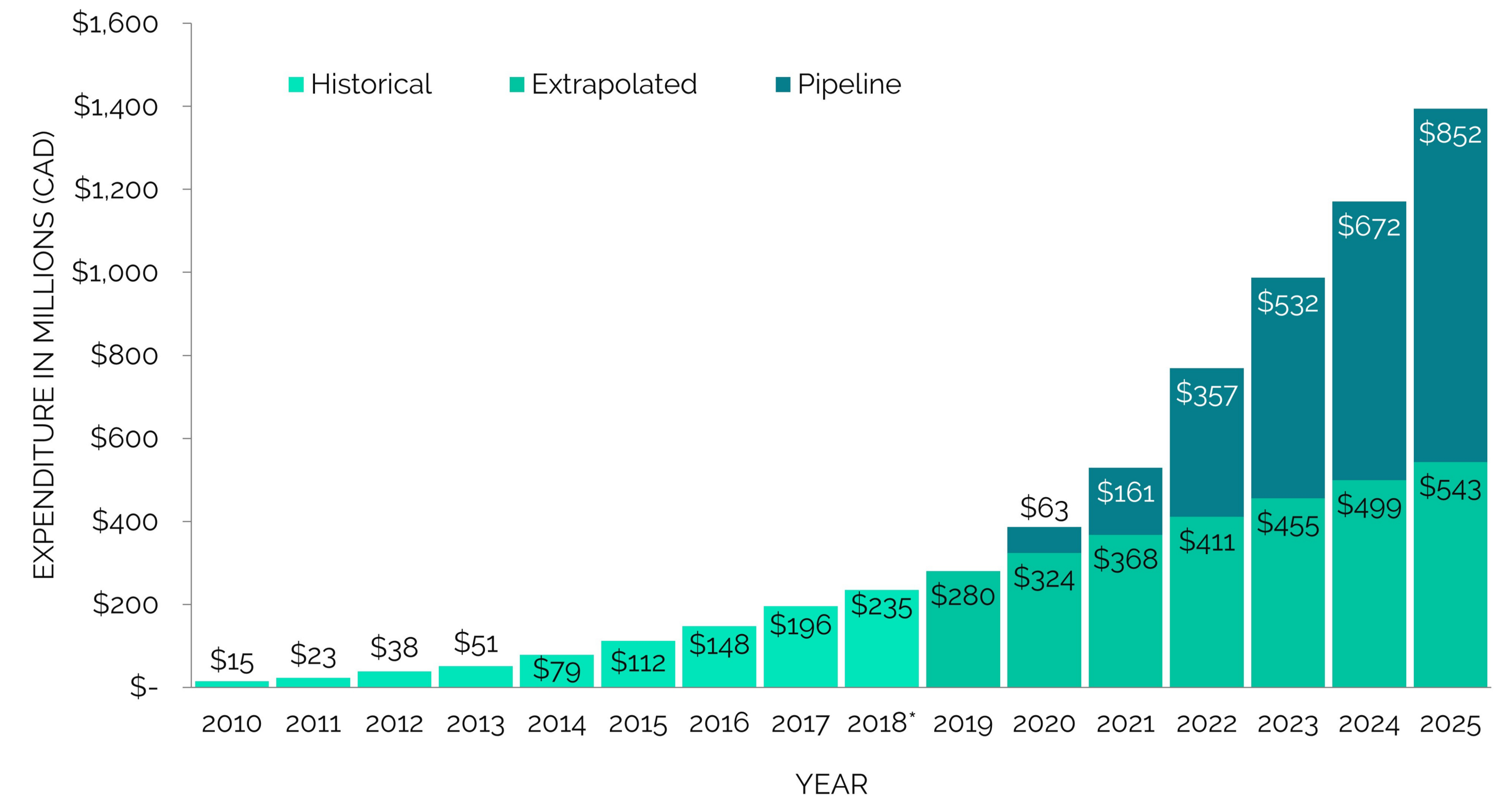


Figure 3. Historical and future (extrapolated + pipeline) public expenditures on DRDs. *Claims data was available until Q3 for 2018 and adjusted to account for Q4

The anticipated annual expenditures for DRDs represent 1.9% of total public drug expenditures in 2019, increasing to 6.5% in 2025 (Figure 4A).

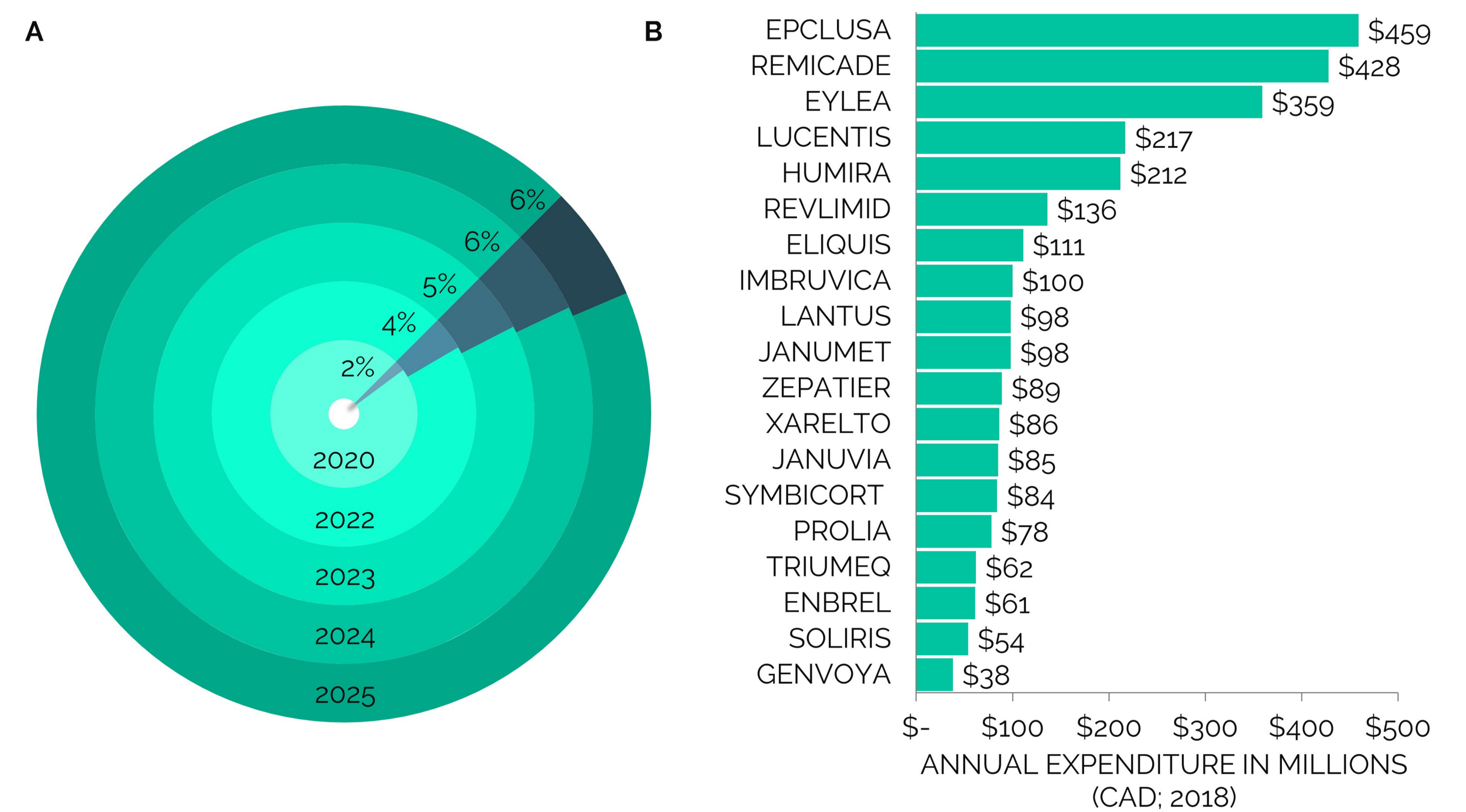


Figure 4A. Public DRD expenditure as a proportion of total public drug expenditure from 2020 to 2025

SA 1 projected an increase to \$543 million in 2025, resulting in 2.5% of total public drug expenditure in 2025; SA 2 projected an increase to \$1,660 million in 2025, resulting in 7.7% of total public drug expenditure in 2025; SA 3 projected an increase to \$2,061 million in 2025, resulting in 9.6% of total public drug expenditure in 2025. In SA 4 (35% confidential price reduction) the public expenditure ranged from \$353 to \$1,530 million, representing 1.6% to 7.1% of total public drug expenditure in 2025.

While the per patient cost of DRDs seems exorbitant compared with the costs of drugs for common conditions, the overall public expenditure for DRDs is minimal when placed in context with other public and consumer expenditures. Of the top 20 public drug expenditures in 2018, only one DRD was part of the list (Figure 4B). The total cost of other medications for non-rare conditions vastly exceeded the cost of this DRD. Similarly, common Canadian government and consumer spending eclipses public DRD spending (Figure 5).

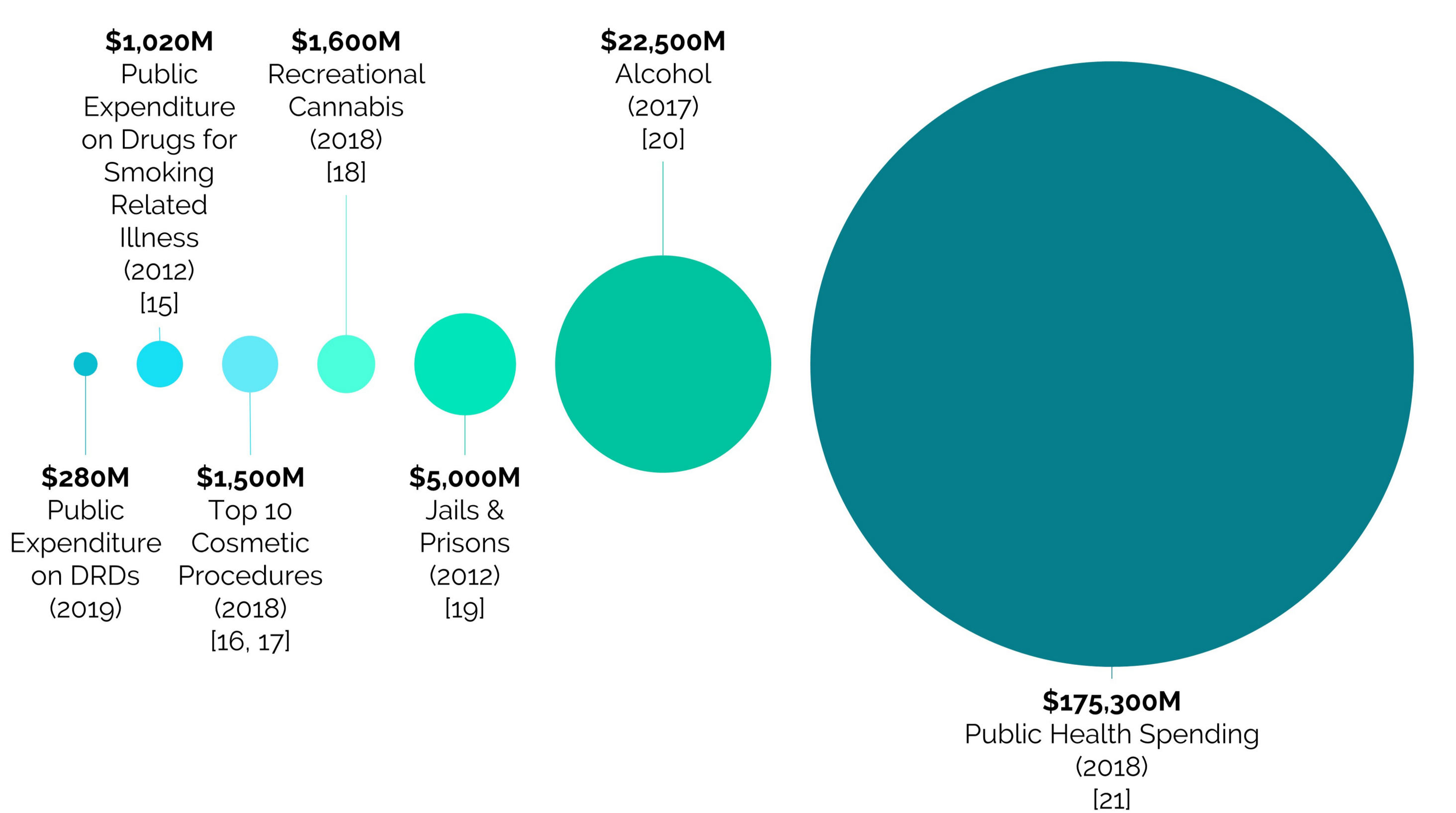


Figure 5. Comparison of the public DRD expenditure to other government and consumer expenditures

DISCUSSION & CONCLUSION

To our knowledge, this is the first study reporting on the potential future total costs of DRDs in Canada using comprehensive projection methods to 2025. Despite the robust methodology, there are limitations with estimating what will occur in the future. Nevertheless, the calculations were conservative for the following reasons:

1. A higher number of new DRDs were projected to enter the market relative to the last decade;
2. The cost of each pipeline DRD was forecasted independently of other drugs for the same indication that might share the same market;
3. The analysis was conducted using public list pricing;
4. The analysis only accounted for drug costs and did not consider other healthcare expenditures that would be avoided with DRDs (i.e., cost-offsets).

Studies examining the cost and value of DRDs are particularly pertinent given the current policy climate in Canada, including the federal government's recent announcement of dedicated funding for a national strategy for DRDs [22]. Concerns regarding unsustainable growth in Canadian public drug expenditures driven by DRDs may not be justified. In the highest cost scenario, where HTA success rates were set to 90%, the total cost of DRDs was 9.6% of the total public drug expenditure in 2025. The results of this study provide important information about the cost of DRDs within the broader context of public health care expenditures and Canadian consumer choices. This analysis can be used to inform future evidence-based discussions about the funding of DRDs in Canada.

REFERENCES

1. Cassidy B. Canadians Affected by Rare Diseases and Disorders: Improving Access to Treatment [Internet]. House of Commons Chambre Des Communes Canada. 2019. Available from: <https://www.ourcommons.ca/Content/Committee/421/HESA/Reports/RP1036306/hesapzz/hesapzz-e.pdf>
2. Orphan designation Overview [Internet]. European Medicines Agency. Available from: <https://www.ema.europa.eu/en/human-regulatory/overview/orphan-designation-overview>
3. What is the "Expense" for Expensive Drugs for Rare Diseases? [Internet]. Available from: <http://www.gprps-osrmb.gc.ca/new.asp?ccid=465&lang=en>
4. Managing the "Expense" in Expensive Drugs for Rare Diseases [Internet]. Symposium cadth.ca. 2019. Available from: <https://symposium.cadth.ca/managing-the-expense-in-expensive-drugs-for-rare-diseases/>
5. Public Health - European Commission [Internet]. Union Register of medicinal products. [cited 2019 Oct 10]. Available from: https://ec.europa.eu/health/documents/community-register/html/reg_od_act.htm?short-a
6. Janusz G, Amegashie W, McIntosh B, Sehgal C, Richter T. Health technology assessment of drugs for rare diseases: insights, trends, and reasons for negative recommendations from the CADTH common drug review. *Orphanet J Rare Dis*. 2020; 15:11254.
7. McCormick JI, Beresuc LD, Tadros N. Common drug review recommendations for orphan drugs in Canada: basis of recommendations and comparison with similar reviews in Quebec, Australia, Scotland and New Zealand. *Orphanet J Rare Dis*. 2018; 13:1327.
8. 2018 Medicines in Development for Rare Diseases: A LIST OF ORPHAN DRUGS IN THE PIPELINE [Internet]. PhRMA. Available from: <https://www.phrma.org/-/media/Project/PhRMA/PhRMA-Orp/PhRMA-Orp/PDF/medicines-in-development-drug-list-rare-diseases.pdf>
9. Jayasundara K, Krishn M, Mamdani M, Hoch JS, Grootendorst P. Differences in Incremental Cost-Effectiveness Ratios for Common Versus Rare Conditions: A Case from Oncology. *Pharmacoeconomics Open*. 2017 Sep; 1(3):67-73.
10. Canadian Drug Claims Database [Internet]. Available from: <http://www.pdci.ca/services/canadian-drug-claims-database/>
11. Schrey G, Milanova T, Hutchings A. Estimating the budget impact of orphan medicines in Europe: 2010 - 2020. *Orphanet J Rare Dis*. 2011 Sep; 27:162.
12. Schildner M, Adenleweh C, Candour A. Budget impact analysis of drugs for ultra-orphan non-oncological diseases in Europe. *Expert Review of Pharmacoeconomics & Outcomes Research*. 2015 Jan; 2(1):171-9.
13. CADTH Common Drug Review (CDR) Reports [Internet]. Common Drug Review (CDR) Reports. Available from: <https://www.cadth.ca/about-cadth/what-we-do/products-services/cdr/reports>
14. Canada's drug spending growth outpaces that for hospitals and doctors | CIHI [Internet]. 2019. Available from: <https://www.cihi.ca/en/canadas-drug-spending-growth-outpaces-that-for-hospitals-and-doctors>
15. Dobrossi A, Bhandari A, Sutherland G, Deth T. The Costs of Tobacco Use in Canada. 2020 [Internet]. Conference Board of Canada. 2017. Available from: https://www.conferenceboard.ca/temp/43c2f54-f670-4d8a-842e-c5927ab97f/g185_Costs-Tobacco-Use_RPT.pdf
16. 2018 National Plastic Surgery Statistics [Internet]. American Society of Plastic Surgeons. 2019. Available from: <https://www.plasticsurgery.org/documents/News/Statistics/2018/plastic-surgery-statistics-report-2018.pdf>
17. American Society of Plastic Surgeons Releases Report Showing National Average of Cosmetic Surgical Fees in 2018 [Internet]. American Society of Plastic Surgeons. 2019. Available from: <https://www.asps.org/press-releases/2019/09/17/american-society-of-plastic-surgeons-releases-report-showing-national-average-of-cosmetic-surgical-fees-in-2018>
18. Canadian Spend \$5.8 Billion on Legal Weed in 2018 Report [Internet]. VICE. 2019. Available from: https://www.vice.com/en_ca/article/439k93/canadians-spent-dollars-billion-on-legal-weed-in-2018-report
19. Financial Facts on Cannabis [Internet]. John Howard Society of Canada. 2019. Available from: <http://johnhoward.ca/blog/financial-facts-canadian-spent-cdr-reports>
20. Control and sale of alcoholic beverages: year ending March 31, 2012 [Internet]. Statistics Canada. Available from: <https://www50.statcan.gc.ca/nr/daily-quotidien/120510/dq120510-eng.htm>
21. Health spending | CIHI [Internet]. 2019. Available from: <https://www.cihi.ca/en/health-spending>
22. Government of Canada Department of Finance. Budget 2019 [Internet]. 2019. Available from: <https://www.budget.gc.ca/2019/docs/themes/pharmaceute-assurance-medicaments-en.html>

