Historical and projected public spending on drugs for rare diseases in Canada between 2010 and 2025

Lech R, Chow G, Mann K, Chattha R, Malmberg C, & Forte L

With support from the Canadian Forum For Rare Disease Innovators

INTRODUCTION

Health Canada defines rare diseases as 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 ≤ 50 persons per 100,000 population [1,2]. Rare diseases affect an estimated 2% to 9% of the Canadian population [1,3], although Canadians only gain access to approximately 60% of globally marketed drugs for rare diseases (DRDs), which is partially related to payer concerns regarding affordability and high per-patient costs [1,4,5]. Unfortunately, limiting access to DRDs dramatically reduces survival and quality of life among patients with rare diseases (and their caregivers), who lack effective treatment options.

OBJECTIVES

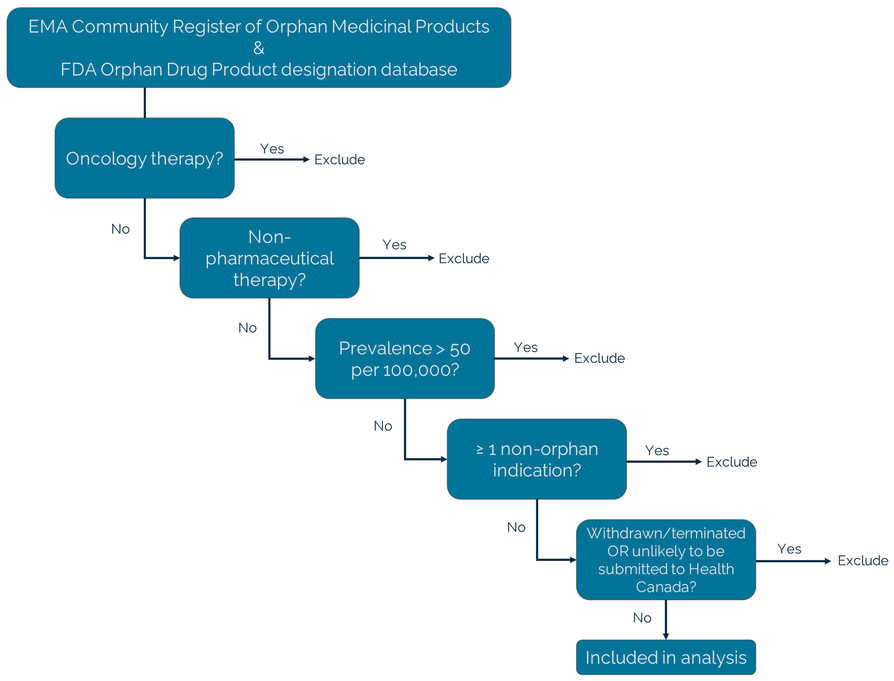
The primary objective was to quantify historical and projected public expenditures on DRDs in Canada between 2010 and 2025. Secondary objectives were to provide context by comparing current DRD spending to: 1) total public drug spending, 2) the top 20 public drug expenditures, and 3) other public and consumer expenditures.

METHODOLOGY

Identification of DRDs

A comprehensive search was performed to identify all publicly funded DRDs and those that could potentially come to market in Canada during 2010–2025. Historical Canadian-marketed DRDs (2010–2020) were identified using the EMA Community Register of Orphan Medicinal Products [6]. Projected pipeline DRDs (2021–2025) were identified using the EMA database and the US Food and Drug Administration (FDA) Orphan Drug Product database. Figure 1 shows the inclusion and exclusion criteria that were used to screen for potential DRDs. Oncology drugs were excluded because rarity does not drive drug prices in oncology [7].

Figure 1. Inclusion and exclusion criteria for screening potential DRDs.



Quantification of DRD Expenditures

Historical expenditures (2010–2020) for publicly funded DRDs in Canada were obtained from a public drug claims database [8]. Freedom of Information requests were made to incorporate reimbursement data from dedicated provincial DRD programs, which were not included in the claims database. All historical drug claim costs were extrapolated in a linear manner from 2010 to 2025. All DRDs were categorized as "orphan" (2 to 50 cases/100,000 population) or "ultra-orphan" (≤2 cases/100,000 population), and pricing data for historical DRDs were used to estimate the prevalence-weighted average per-patient costs ($103,774/year and $356,279/year, respectively) from 2021 to 2025.

To simplify the analyses, health care expenditures that could be avoided with DRD treatment (e.g., to manage comorbidities and adverse events) was not considered, although this would normally be incorporated into a budget impact analysis [9]. Future expenditures for pipeline DRDs expected to reach the Canadian market were based on the prevalence-weighted average costs and the following assumptions:

- Projected drug launch year [assumption]: from 2021 (pCPA negotiations completed) to 2025 (active phase 2/3 trial)
- Regulatory success rate from phase 3 trial to FDA/EMA approval [10]: 66.3% approved, 33.7% rejected
- Canadian approval post-FDA/EMA [11,12]: 16% (year 1), 30% (year 2), 40% (year 3), 50% (year 4), 60% (year 5)
- Canadian HTA success rate among DRDs [13,14]: 69.15% positive, 30.85% do not list
- Market penetration in Canada [15]: 10% (year 1), 12% (year 2), 15% (year 3), 20% (year 4), 25% (year 5)
- Public/private coverage split [assumption]: 60% public, 40% private
- Product launch half-cycle correction [assumption]: 50% of eligible market captured in launch year.

The base case and scenario analyses (SAs) were defined as:

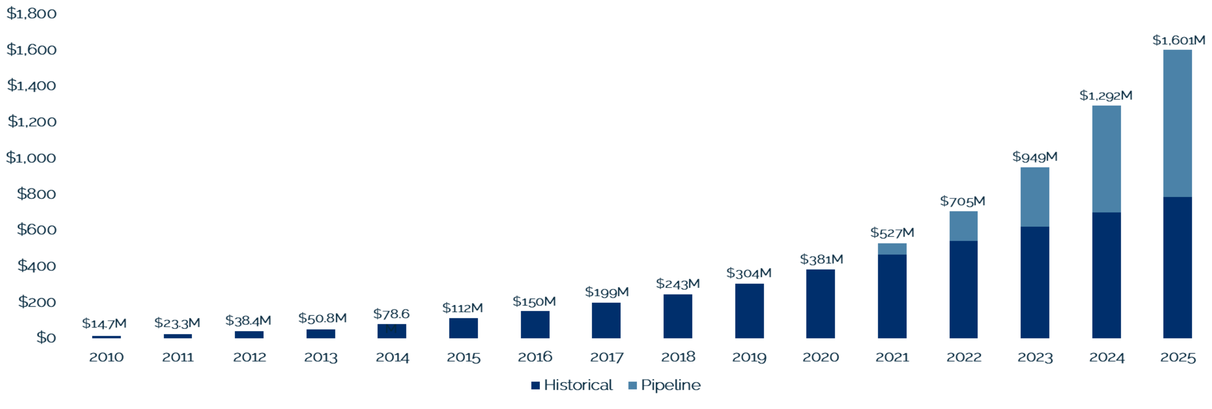
Base case: extrapolation of historical DRD costs + projection of pipeline DRDs
SA 1: extrapolation of only historical DRD costs without costs for pipeline DRDs
SA 2: applying a 35% rebate to projected costs (based on confidential negotiations)
SA 3: assuming a higher HTA success rate (90%)
SA 4: increasing the average per-patient ultra orphan drug cost to $435,000/year
SA 5: increasing the average per-patient orphan drug cost to $175,000/year.

Total costs for drugs with the 20 highest Canadian public expenditures were collected from historical claims data. Total public expenditures for all drugs (Canadian Institute for Health Information data, 2014 to 2018) were projected in a linear manner to 2025 [16].

RESULTS

We identified 554 potentially eligible DRDs, although only 42 historical DRDs and 122 pipeline DRDs were eligible after applying the inclusion and exclusion criteria. The most common exclusions were based on withdrawal of orphan drug status (by the EMA), non-orphan indications (e.g., malaria), and oncology drugs. Public DRD spending grew from $14.8 million in 2010 (11 DRDs) to $380.9 million in 2020, $527.6 million in 2021, and $1.6 billion in 2025 (164 DRDs) (Figure 2).

Figure 2. Estimated public spending on historical and pipeline DRDs between 2010 and 2025



Total public drug spending increased from $11.4 billion in 2014 to $14.5 billion in 2018. Projected DRD spending increased from 3.2% of $16.5 billion total public drug spending in 2021 to 8.3% of $19.4 billion in 2025 (Figure 3). These projections are likely overestimates, as they ignore health outcome-related cost offsets and confidential discounts.

Figure 3. Spending on DRDs as a proportion of total public drug spending

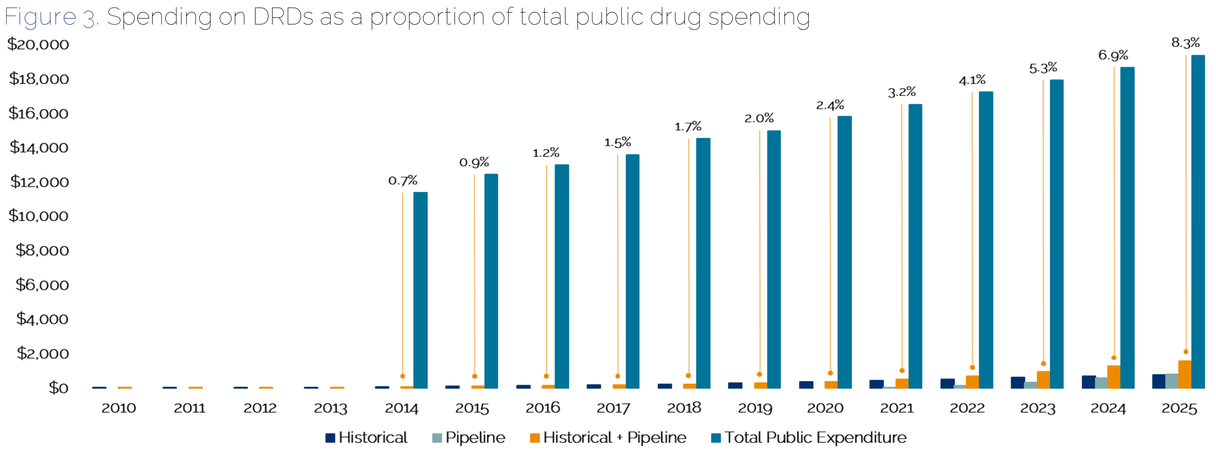
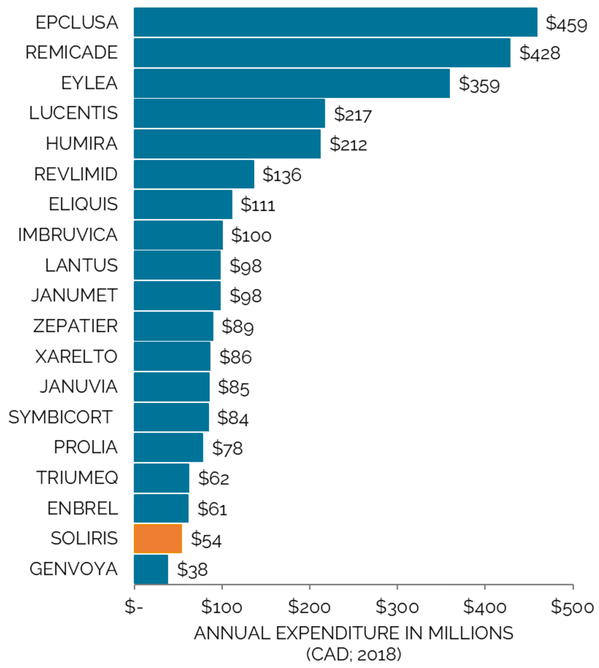


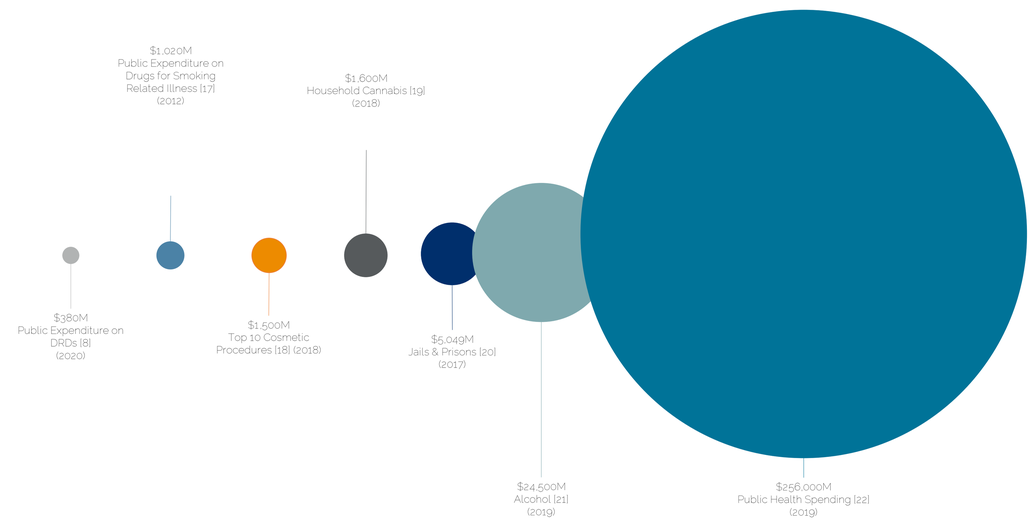
Figure 4. Top 20 public drug expenditures in Canada.



The scenario analyses showed that the projected percentage of total public drug spending on DRDs in 2025 ranged from 4.0% to 10.3%. The lowest scenario was SA 1 (only historical DRD costs without costs for pipeline DRDs), which projected an increase in DRD spending to $782.8 million in 2025 (4.0% of total public drug spending). The highest scenario was SA 5 (increasing the average per-patient orphan drug cost to $175,000/year), which projected an increase to $2.00 billion (10.3% of total public drug spending).

The per-patient cost of DRDs may seem exorbitant relative to costs of drugs for common conditions. However, patients with rare diseases have few or no treatment options to improve their quality of life and survival. In addition, public spending on DRDs is minimal compared to other public and consumer expenditures. For example, only 1 DRD (SOLIRIS™) was included in the top 20 public drug expenditures during 2018 (Figure 4), and the costs of other common drugs vastly exceeded that of SOLIRIS™. Similarly, other public and consumer expenditures eclipsed public DRD spending (Figure 5).

Figure 5. Comparison of public DRD spending to other government and consumer expenditures [8,17–22].



DISCUSSION & CONCLUSION

We believe this is the first study to comprehensively project Canadian public DRD spending until 2025. Although future projections are subject to various limitations, our projections are likely conservative because: 1) we estimated a higher number of new DRDs entering the market (vs. during the last decade), 2) the cost of each pipeline DRD was forecasted independently of other drugs that might share the market for the same indication, 3) the analyses used public list pricing (i.e., ignored confidential manufacturer discounts), and 4) the analyses did not consider other expenditures that might be avoided with DRD use (i.e., cost offsets).

Studies examining DRD costs and values are pertinent given the current policy climate in Canada, including the federal government's announcement of funding for a national DRD strategy [23]. The results of this analysis suggest that concerns regarding unsustainable growth in Canadian public spending on DRDs may not be justified. Our base case projection was 8.3% of public drug spending on DRDs in 2025, with scenario analyses showing proportions of 4.0% to 10.3%. Therefore, projected DRD spending is in line with the proportion of Canadians afflicted with rare diseases (2% to 9%) [1,3]. Furthermore, our projections are likely overestimates, as they do not consider manufacturer discounts or health outcome-related cost offsets, which influence the budget impact of DRDs. Finally, limiting DRD access is not aligned with patient or societal values and creates unfair treatment gaps in Canada's health care system.

REFERENCES

[1] Casey B. Canadians Affected by Rare Diseases and Disorders: Improving Access to Treatment. House of Commons Chambre Des Communes Canada. 2019. Available from: https://bit.ly/3tKUAwk. [2] European Medicines Agency. Orphan designation: Overview. Available from: https://bit.ly/3kd7mPD. [3] Divino V, DeKoven M, Kleinrock M, Wade RL, Kim T, Kaura S. Pharmaceutical expenditure on drugs for rare diseases in Canada: a historical (2007–13) and prospective (2014–18) MIDAS sales data analysis. Orphanet J Rare Dis. 2016;11:68. [4] Lungu E. What is the "Expense" for Expensive Drugs for Rare Diseases? Patented Medicine Prices Review Board. 2019. Available from: https://bit.ly/3nFRPLM. [5] Potashnik T. Managing the "Expense" in Expensive Drugs for Rare Diseases. Patented Medicine Prices Review Board. 2019. Available from: https://bit.ly/2VXMmFL. [6] Public Health - European Commission. Union Register of medicinal products. 2020. Available from: https://bit.ly/3lzheUP. [7] Jayasundara K, Krahn 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;1(3):167–73. [8] PDCI Market Access Inc. Canadian Drug Claims Database. 2020. Available from: https://bit.ly/3AgIsqo. [9] Schlander M, Adarkwah CC, Gandjour A. Budget impact analysis of drugs for ultra-orphan non-oncological diseases in Europe. Expert Review of Pharmacoeconomics & Outcomes Research. 2015 Jan 2;15(1):171–9. [10] Wong CH, Siah KW, Lo AW. Estimation of clinical trial success rates and related parameters. Biostatistics. 2019;20:273–86. [11] Rawson SB. Fewer new drug approvals in Canada: early indication of unintended consequences from new PMPRB regs? 2020. Available from: https://bit.ly/3kgEvLL. [12] Canadian Organization for Rare Disorders. Key facts. Available from: https://www.raredisorders.ca/about-cord. [13] McCormick JI, Beresciu 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;13:27. [14] Patient Access Solutions. Analysis of positive CADTH recommendations from 2018 to 2021. Data on File. [15] Schey C, Milanova T, Hutchings A. Estimating the budget impact of orphan medicines in Europe: 2010 - 2020. Orphanet J Rare Dis. 2011;6:62. [16] Canadian Institute for Health Information. Canada's drug spending growth outpaces that for hospitals and doctors. 2019. Available from: https://bit.ly/3Enod8W. [17] The Conference Board of Canada. The Costs of Tobacco Use in Canada, 2012. 2017. Available from: https://bit.ly/3kdixt6. [18] American Society of Plastic Surgeons. American Society of Plastic Surgeons Releases Report Showing National Average of Cosmetic Surgical Fees in 2016. 2019. Available from: https://bit.ly/3ErgSbc. [19] Statistics Canada. Table 36-10-0603-01. Household final consumption expenditure of cannabis, inactive (x 1,000,000). 2019. Available from: https://bit.ly/3kdtbQz. [20] Statistics Canada. Table 6 Operating expenditures of the adult correctional system, by jurisdiction, 2017/2018. Available from: https://bit.ly/3kgSESi. [21] Statistics Canada. Control and sale of alcoholic beverages, year ending March 31, 2020. Available from: https://bit.ly/3AujR0O. [22] Canadian Institute for Health Information. Health spending. Available from: https://www.cihi.ca/en/health-spending. [23] Government of Canada Department of Finance. Budget 2019. 2019 Available from: https://bit.ly/3CgdHr.