Budget Impact of Drugs for Rare Disease in Canada and the Potential Impact on Reimbursement

Lindy Forte
Sr. VP, Market Access & Pricing
EVERSANA Canada

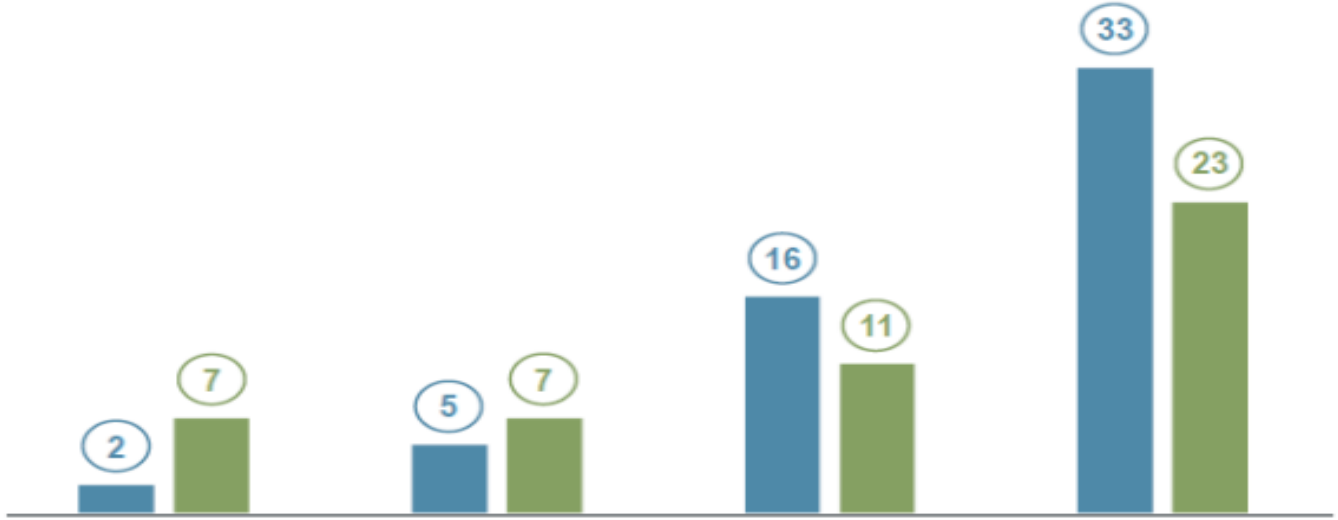
Development of DRDs has been increasing year over year

The pace of EDRD approvals in Canada has increased over the past decade

Until 2010, few medicines in Canada met the criteria to be an EDRD. Over the past decade, however, the number of EDRDs gaining market approval has grown substantially. The total number of EDRDs more than doubled from 2011 to 2015 and doubled again from 2016 to 2020 to reach 104.

The large number of EDRDs, especially oncology medicines, coming to market in recent years has been a major driver of the overall growth in EDRD sales.

FIGURE 1.1 EDRD approvals in Canada by year of Notice of Compliance, oncology and non-oncology, as of 2020



Cumulative no. of EDRDs

	Pre-2006	2006 to 2010	2011 to 2015	2016 to 2020
Oncology	2	7	23	56
Non-oncology	7	14	25	48
Total	**9**	**21**	**48**	**104**

PMPRB. Expensive Drugs for Rare Diseases: Trends and International Comparisons, 2011–2020. January 2022. Available on the PMPRB website.

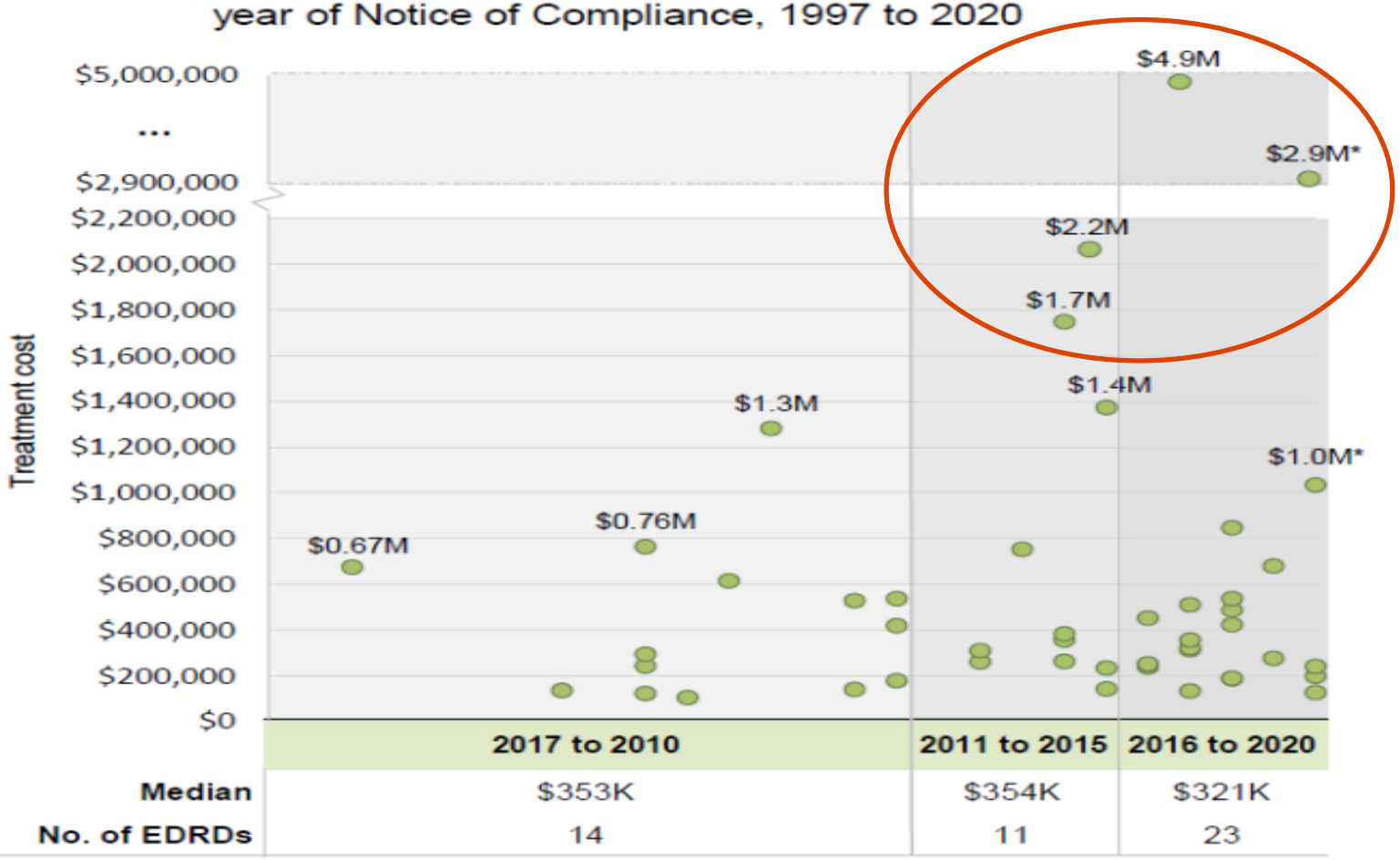
© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Annual per patient treatment costs of DRDs has been increasing

Non-oncology EDRDs with multimillion-dollar treatment costs have gained approval in the past decade

Since the start of the decade, a cohort of new medicines with multimillion-dollar annual costs per patient have gained market approval. Despite this, numerous launches in the $100,000 to $500,000 range resulted in a lower median for new launches than in previous periods.

FIGURE 1.3 Annual treatment costs for non-oncology EDRDs in Canada by year of Notice of Compliance, 1997 to 2020



* One-time treatment.

Data source: CADTH; Health Canada Drug Product Database; product monographs.

What is the Future Expected Cost of DRD Funding in Canada?

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Lech *et al.* *Orphanet Journal of Rare Diseases.* *October 2022*

With support from the Canadian Forum For Rare Disease Innovators

RARE i

Lech et al. Orphanet Journal of Rare Diseases (2022) 17:371
https://doi.org/10.1186/s13023-022-02534-z

Orphanet Journal of Rare Diseases

RESEARCH Open Access

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

Richard Lech, Gideon Chow, Kamalpreet Mann, Patrick Mott*, Christine Malmberg and Lindy Forte

Abstract

Objective: Rare diseases are life-threatening, debilitating, or serious chronic conditions that affect < 50/100,000 people. Canadians can only access approximately 60% of drugs for rare diseases (DRDs), which is partially related to high per-patient costs and payers' affordability concerns. However, limiting access to DRDs can reduce survival and quality of life among patients and caregivers. Therefore, we projected Canadian non-oncology DRD spending relative to total public drug spending to provide perspective for decision makers.

Methods: Candidate historical (2010–2020) and pipeline (2021–2025) Canadian-marketed non-oncology DRDs were identified using definitions from the European Medicines Agency and the US Food and Drug Administration databases. Inclusion and exclusion criteria were applied to identify eligible DRDs. Public payer claims data, prevalence rates, regulatory, and health technology assessment factors were used to project DRD spending in relation to total Canadian public drug spending.

Results: We included 42 historical DRDs and 122 pipeline DRDs. Public spending on DRDs grew from $14.8 million in 2010 (11 DRDs) to $380.9 million in 2020, then a projected $527.6 million in 2021 (59 potential DRDs) and $1.6 billion in 2025 (164 potential DRDs). Projected DRD spending increased from 3.2% of $16.5 billion public drug spending in 2021 to 8.3% of $19.4 billion in 2025. These projections do not include confidential manufacturer discounts, health outcome-related offsets, or additional safety-related costs.

Conclusions: Projected DRD spending shows robust growth but remains a fraction of total public drug spending. Limiting DRD access because of this growth is not aligned with Canadian patient or societal values. Given the renewed interest in a Canadian DRD framework, our results may help guide discussions that aim to balance control of public drug spending with the well-being of patients with rare diseases.

Keywords: Canada, Drugs for rare diseases, Orphan drugs, Drug funding, Orphan diseases, Patient access, Public spending, Rare disease

Introduction

Health Canada defines rare diseases as life-threatening, debilitating, or serious and chronic conditions that affect a small number of individuals (< 50 cases per 100,000 population) [1]. While each rare disease has a small patient population, together rare diseases affect an estimated 2–9% of the general population [1, 2]. Rare diseases are often fatal and can have a devastating effect on life expectancy and quality of life, especially as a large proportion of rare diseases occur during childhood [3, 4]. Moreover, an Italian registry study suggested that rare diseases accounted for 4.2% of years of life lost in the general population, which was higher than the proportions

*Correspondence: Patrick.mott@eversana.com
CRG-EVERSANA Canada Inc., 219 Dufferin St., Suite 210B, Toronto, ON M6K 3J1, Canada

BMC

© The Author(s) 2022. **Open Access** This article is licensed under a Creative Commons Attribution 4.0 International License, which permits use, sharing, adaptation, distribution and reproduction in any medium or format, as long as you give appropriate credit to the original author(s) and the source, provide a link to the Creative Commons licence, and indicate if changes were made. The images or other third party material in this article are included in the article's Creative Commons licence, unless indicated otherwise in a credit line to the material. If material is not included in the article's Creative Commons licence and your intended use is not permitted by statutory regulation or exceeds the permitted use, you will need to obtain permission directly from the copyright holder. To view a copy of this licence, visit http://creativecommons.org/licenses/by/4.0/. The Creative Commons Public Domain Dedication waiver (http://creativecommons.org/publicdomain/zero/1.0/) applies to the data made available in this article, unless otherwise stated in a credit line to the data.

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Objective

To quantify and contextualize the historical and projected total future public expenditures on DRDs in Canada.

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Abbreviations: DRD = drug for rare disease

Methodology

© 2022 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Screened all Orphan Drugs from EMA and FDA databases

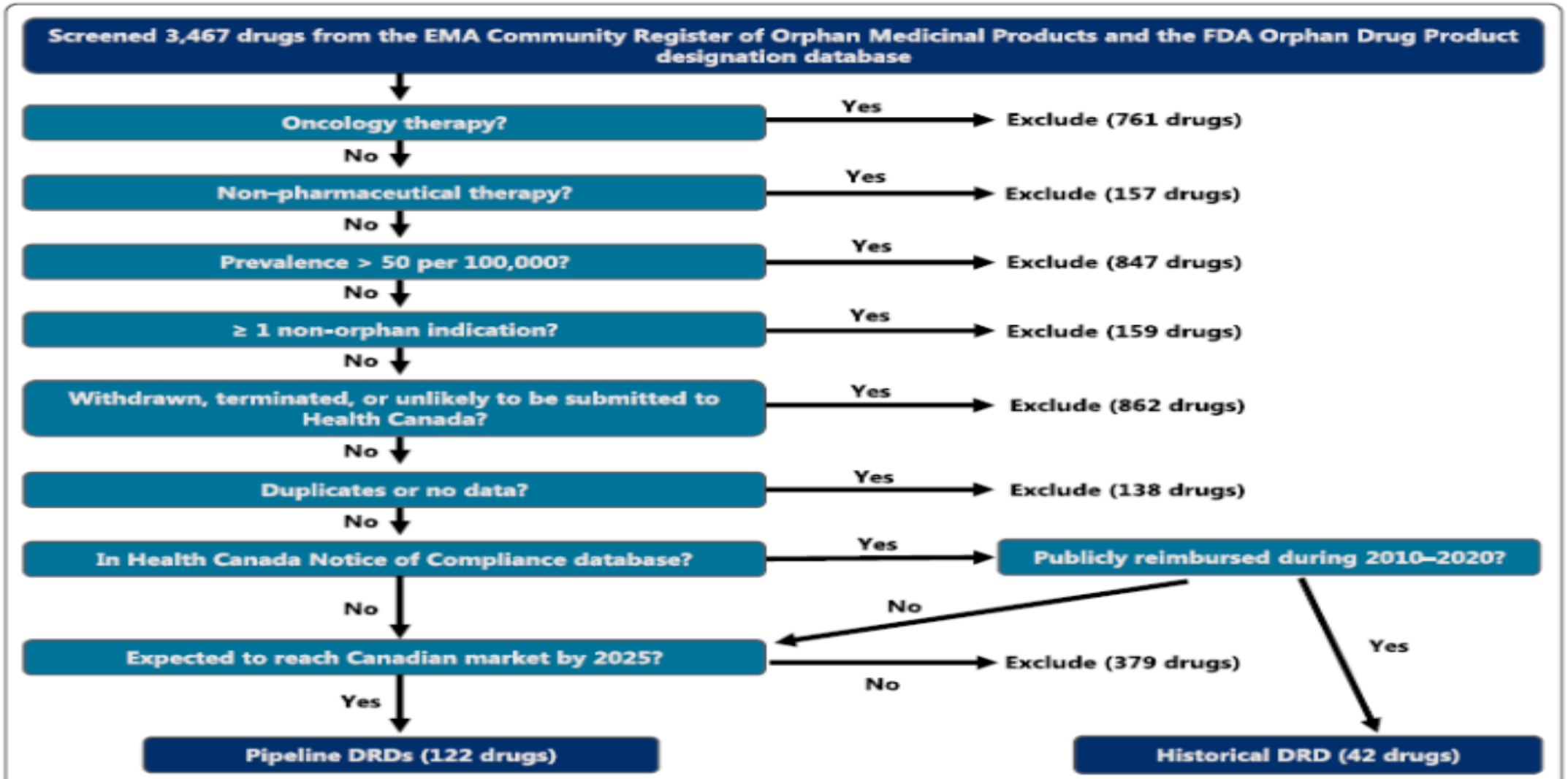


Fig. 1 Flowchart identifying historical and pipeline drugs for rare diseases in Canada. As Canada does not have an official framework for drugs for rare diseases (DRDs), candidate DRDs were identified using the European Medicines Agency (EMA) Community Register of Orphan Medicinal Products, the US Food and Drug Administration (FDA) Orphan Drug Product designation database, and the Health Canada (HC) Notice of Compliance database. Various inclusion and exclusion criteria were applied to determine whether a DRD would reach the Canadian market by 2025.

Assumptions regarding future public DRD spending in Canada

Assumptions were based on historical data:

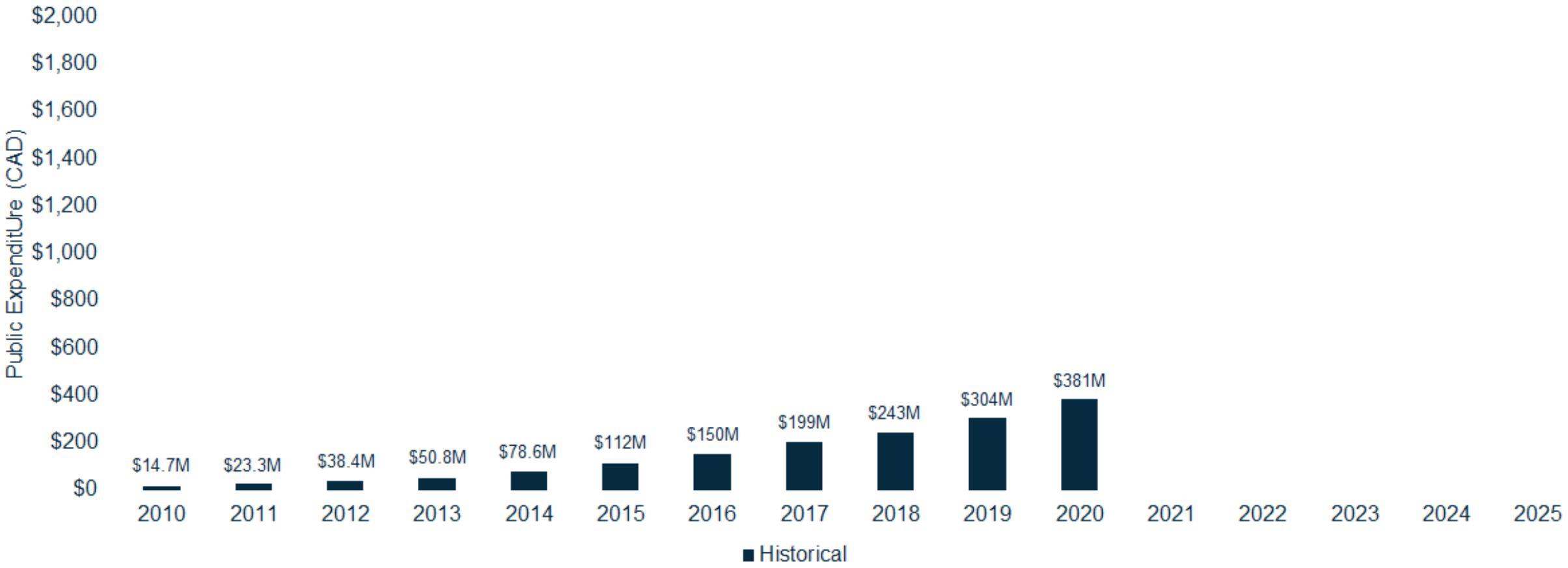
- Rate of success with Health Canada approval of DRDs
- Rate of success with CADTH/INESSS recommendations for DRDs
- Rate of success and timing of pCPA negotiation and jurisdictional reimbursement for DRDs
- Launch price for rare and ultra-rare DRDs
- Treatment rates over time
- Public sector only
- Non-oncology DRDs

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Results

© 2022 EVERSANA. All Rights Reserved. CONFIDENTIAL.

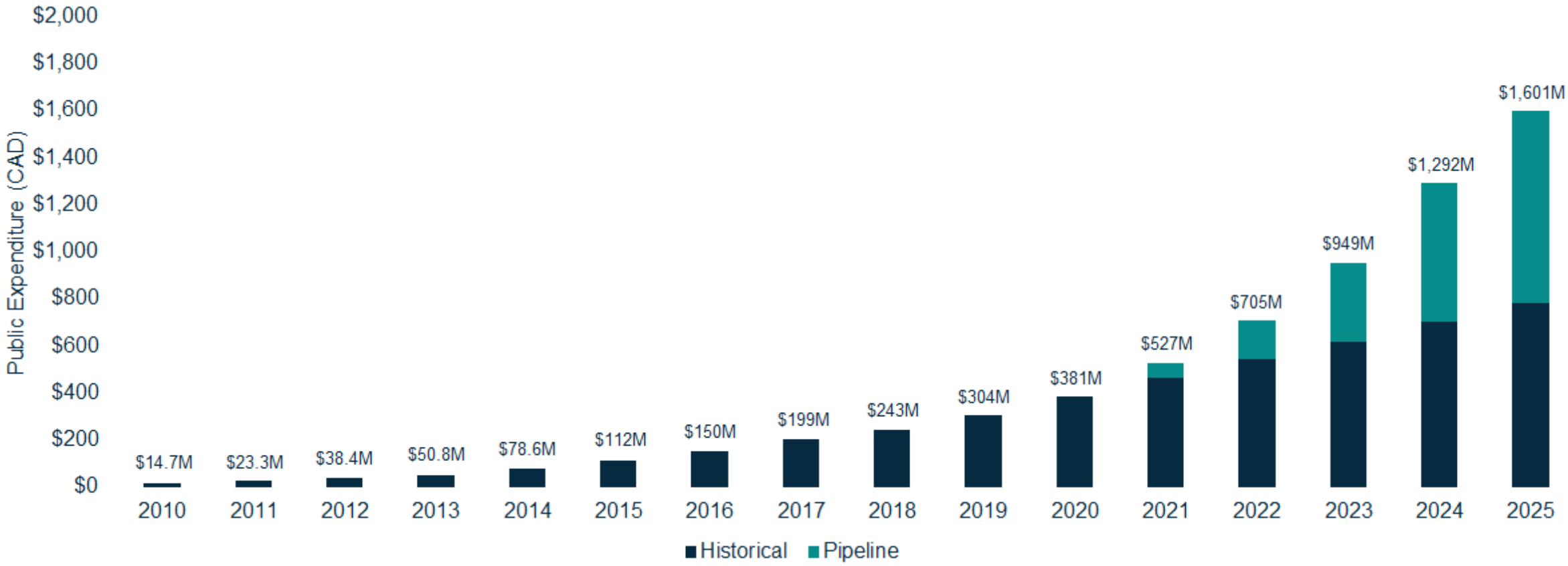
Historical DRD expenditure (2010 to 2020)



Abbreviations: DRD = drug for rare disease.

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

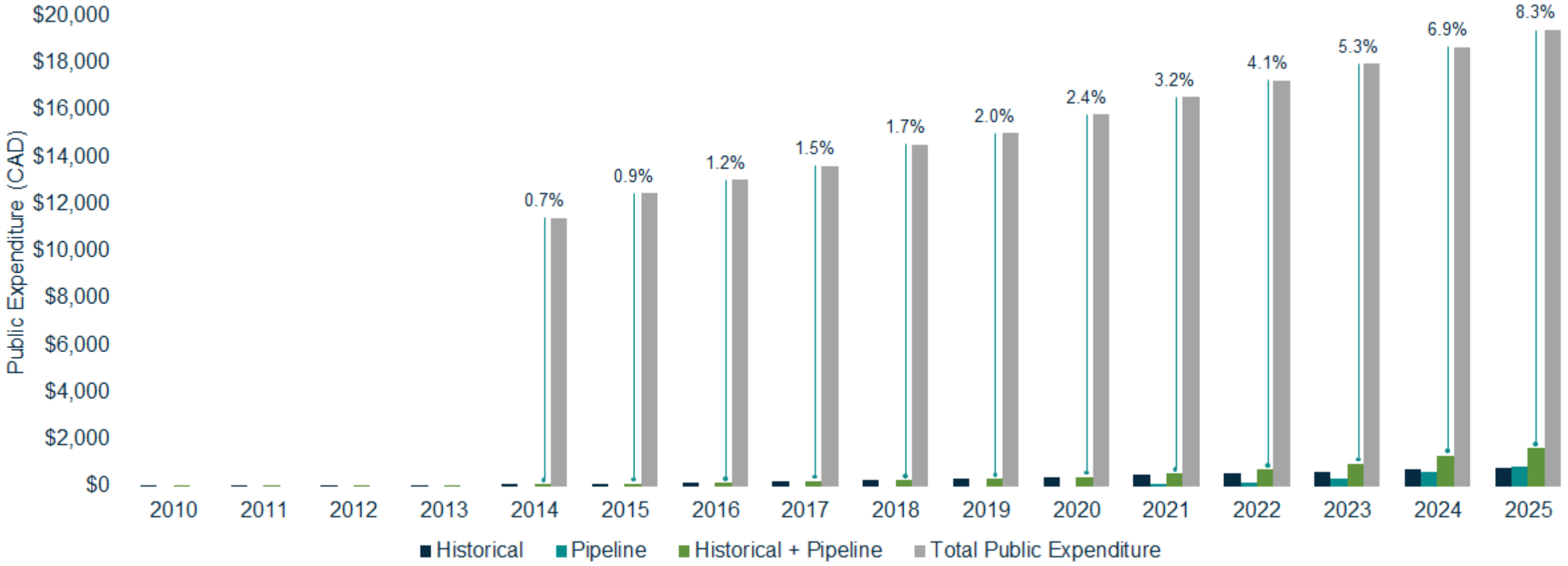
DRD expenditure forecast (2021 to 2025)



Abbreviations: DRD = drug for rare disease.

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

DRD expenditure forecast vs. total public drug expenditure forecast

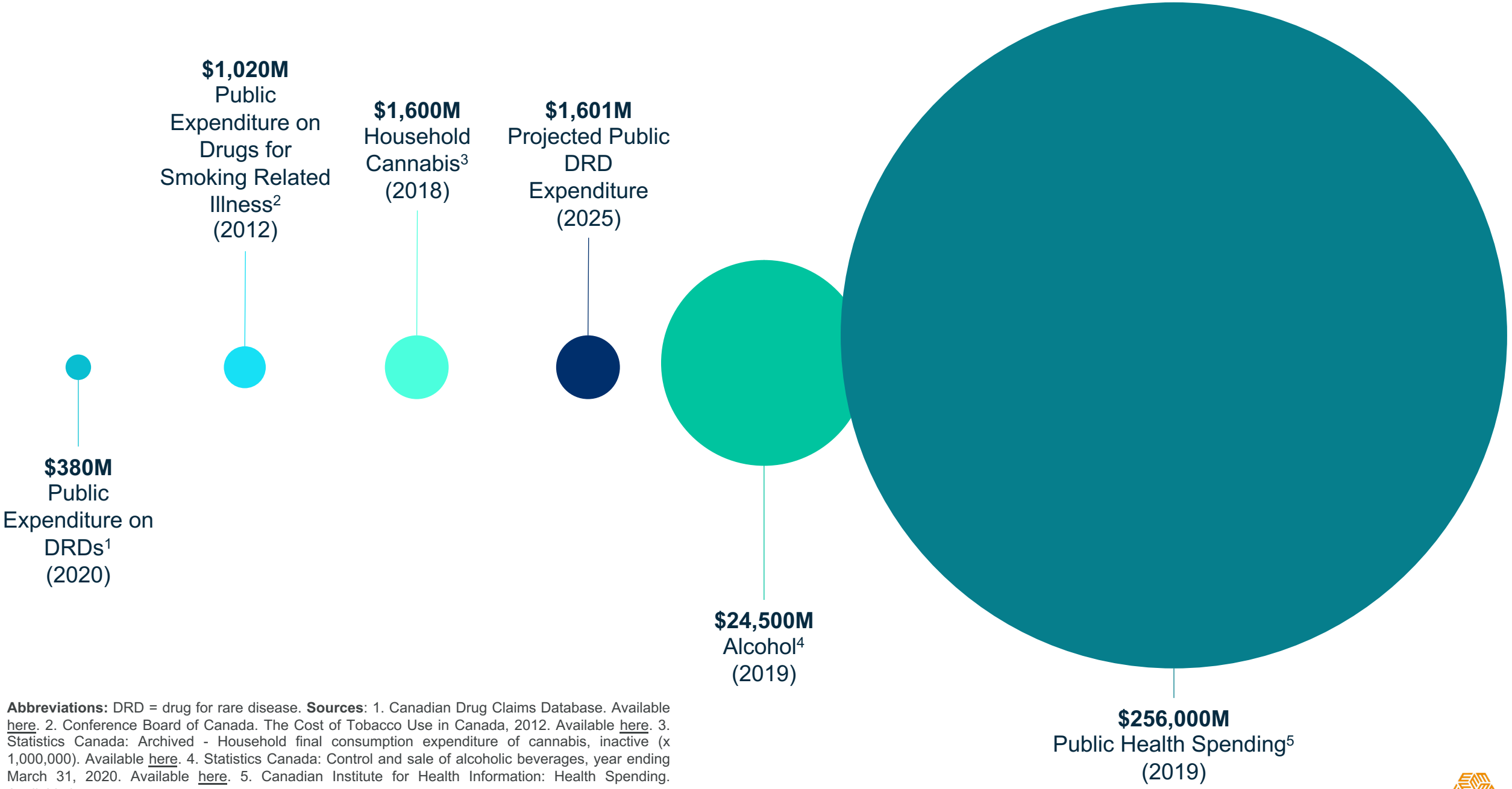


Abbreviations: DRD = drug for rare disease.

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

How does the current cost of DRDs compare to other Canadian expenditures?

© 2022 EVERSANA. All Rights Reserved. CONFIDENTIAL.



Abbreviations: DRD = drug for rare disease. **Sources**: 1. Canadian Drug Claims Database. Available here. 2. Conference Board of Canada. The Cost of Tobacco Use in Canada, 2012. Available here. 3. Statistics Canada: Archived - Household final consumption expenditure of cannabis, inactive (x 1,000,000). Available here. 4. Statistics Canada: Control and sale of alcoholic beverages, year ending March 31, 2020. Available here. 5. Canadian Institute for Health Information: Health Spending. Available here.

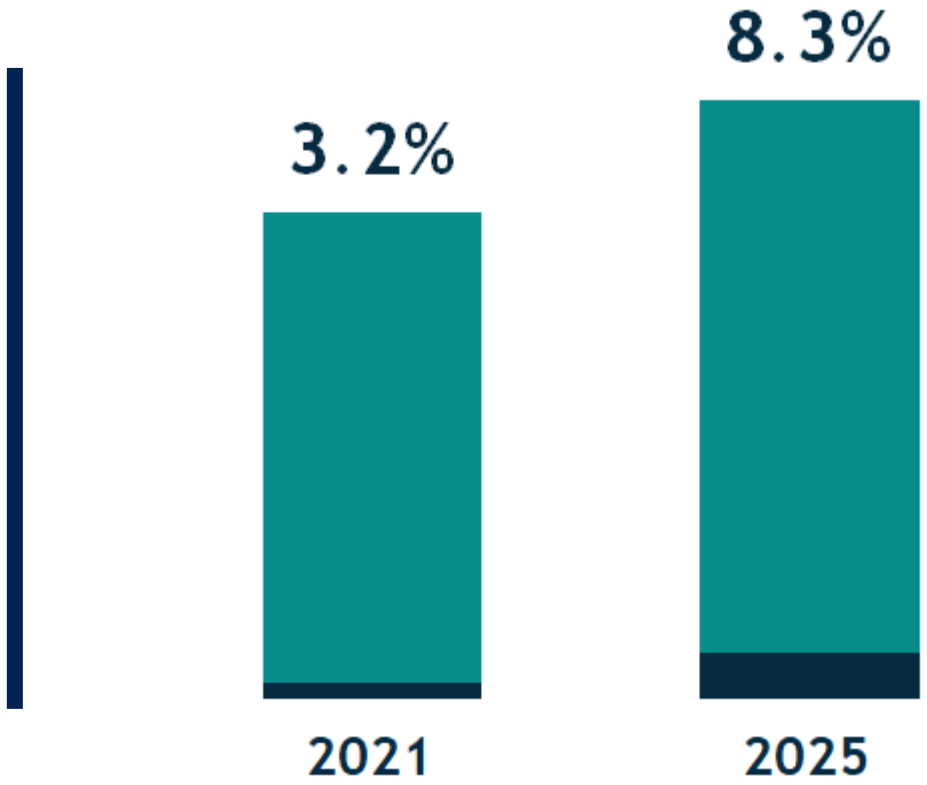
Conclusions

© 2022 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Conclusions

DRD expenditure are projected to continue to represent a small proportion of total pharmaceutical expenditure:

- **3.2% of $16.5B in 2021**
- **8.3% of $19.4B in 2025**



Abbreviations: DRD = drug for rare disease.

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

How do the results compare to other sources of cost projection?

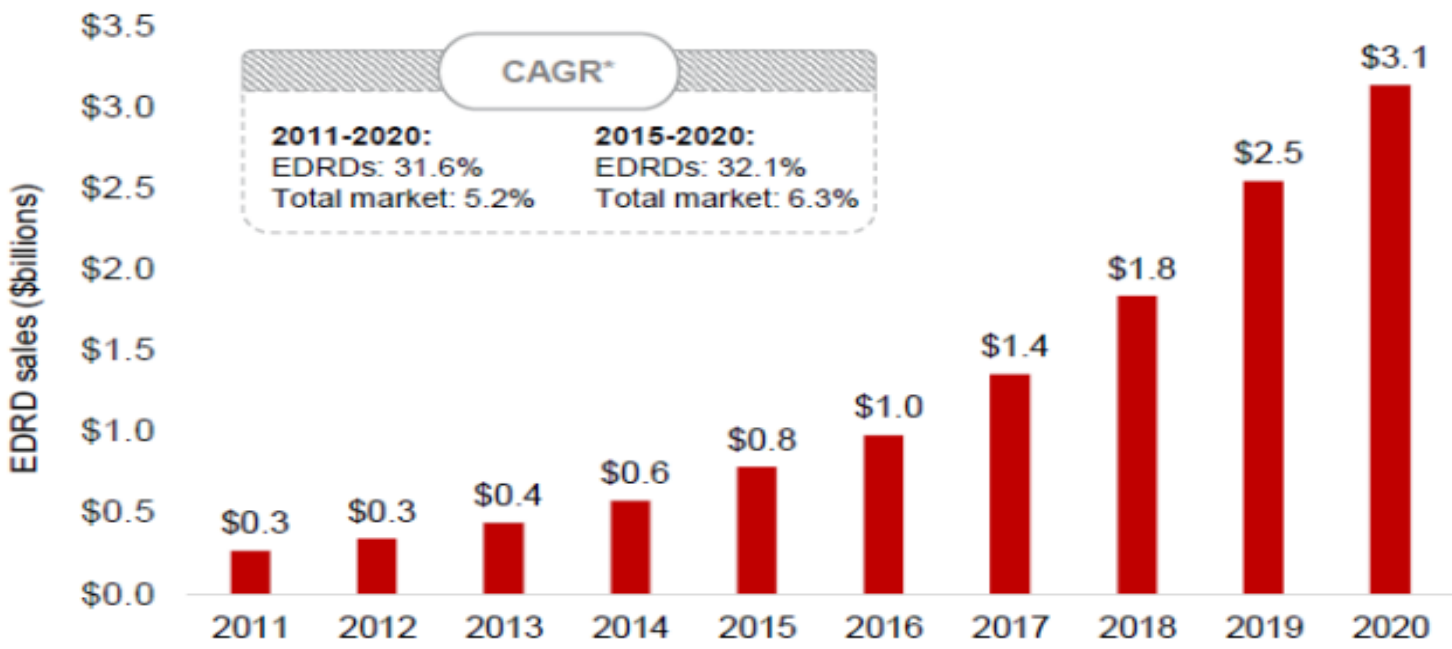
Sales of EDRDs in Canada have grown rapidly since 2016

A combination of new market entries and growth of existing medicines caused sales of EDRDs to increase at a compound annual growth rate of 31.6% between 2011 and 2020, greatly outpacing the overall pharmaceutical market.

In the latter half of the decade, compound annual growth was 32.1%, resulting in sales of $3.1 billion in 2020, more than one tenth of the total market.

* Compound annual growth rate.

FIGURE 2.1 Sales of EDRDs in Canada, 2011 to 2020



	2011	2012	2013	2014	2015	2016	2017	2018	2019	2020
Annual growth rate	-	28.1%	29.5%	30.6%	36.1%	25.3%	38.5%	35.3%	38.8%	23.2%
Share of Canadian pharmaceutical market	1.4%	1.7%	2.2%	2.8%	3.5%	4.2%	5.4%	7.0%	9.1%	10.4%
Share of patented medicine sales	2.2%	2.8%	3.4%	4.0%	5.2%	6.3%	7.8%	11.7%	15.1%	17.4%

PMPRB. Expensive Drugs for Rare Diseases: Trends and International Comparisons, 2011–2020. January 2022. Available on the PMPRB website.

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Can We Ensure Continued DRD Funding While Improving Time to Access?

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Challenges securing positive recommendations due to level of evidence

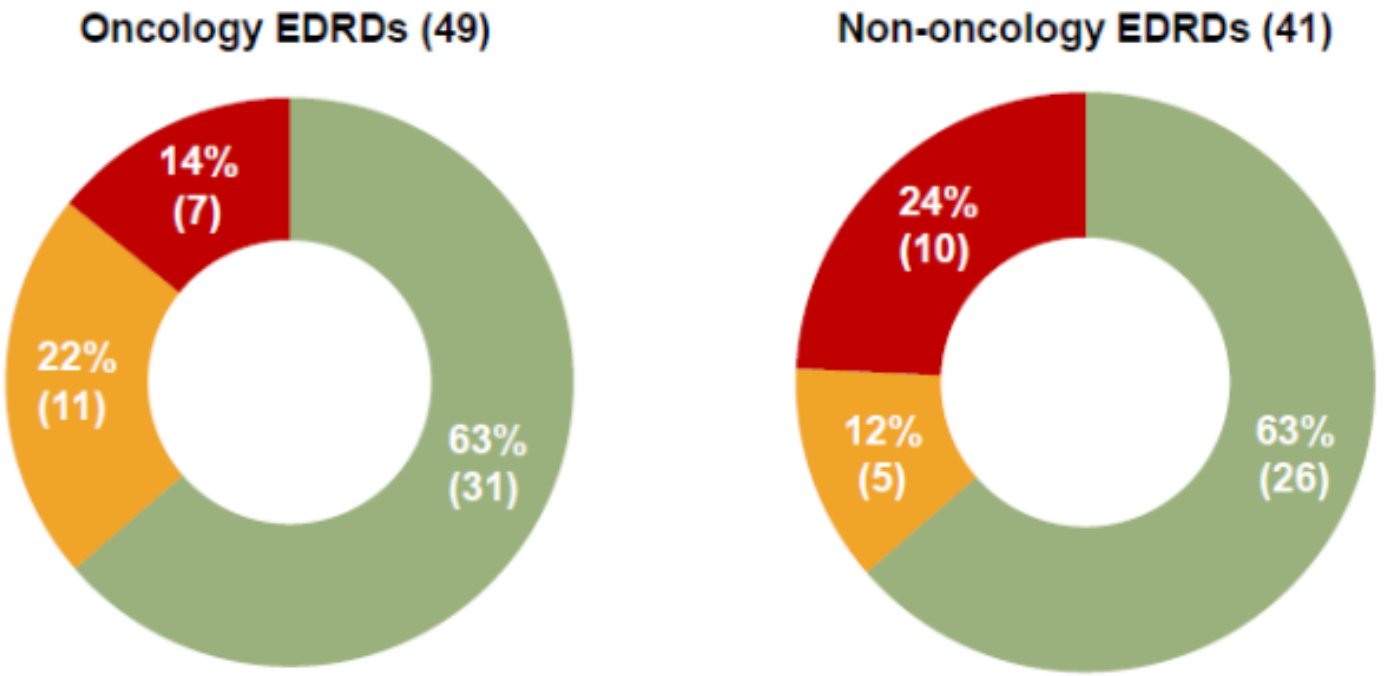
When positive, price recommendations are required

CADTH reimbursement reviews for EDRDs typically recommend price reduction

While the majority of oncology and non-oncology EDRDs have received at least one positive reimbursement recommendation from CADTH, it is almost always conditional on an improvement in cost-effectiveness over the manufacturer-submitted price.

Less common are medicines that received a mix of positive and negative recommendations for different indications, or solely negative recommendations to not reimburse.

FIGURE 1.5 CADTH reimbursement recommendations* for oncology and non-oncology EDRDs, as of March 2021



Each EDRD is counted once and classified according to its CADTH review results.

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

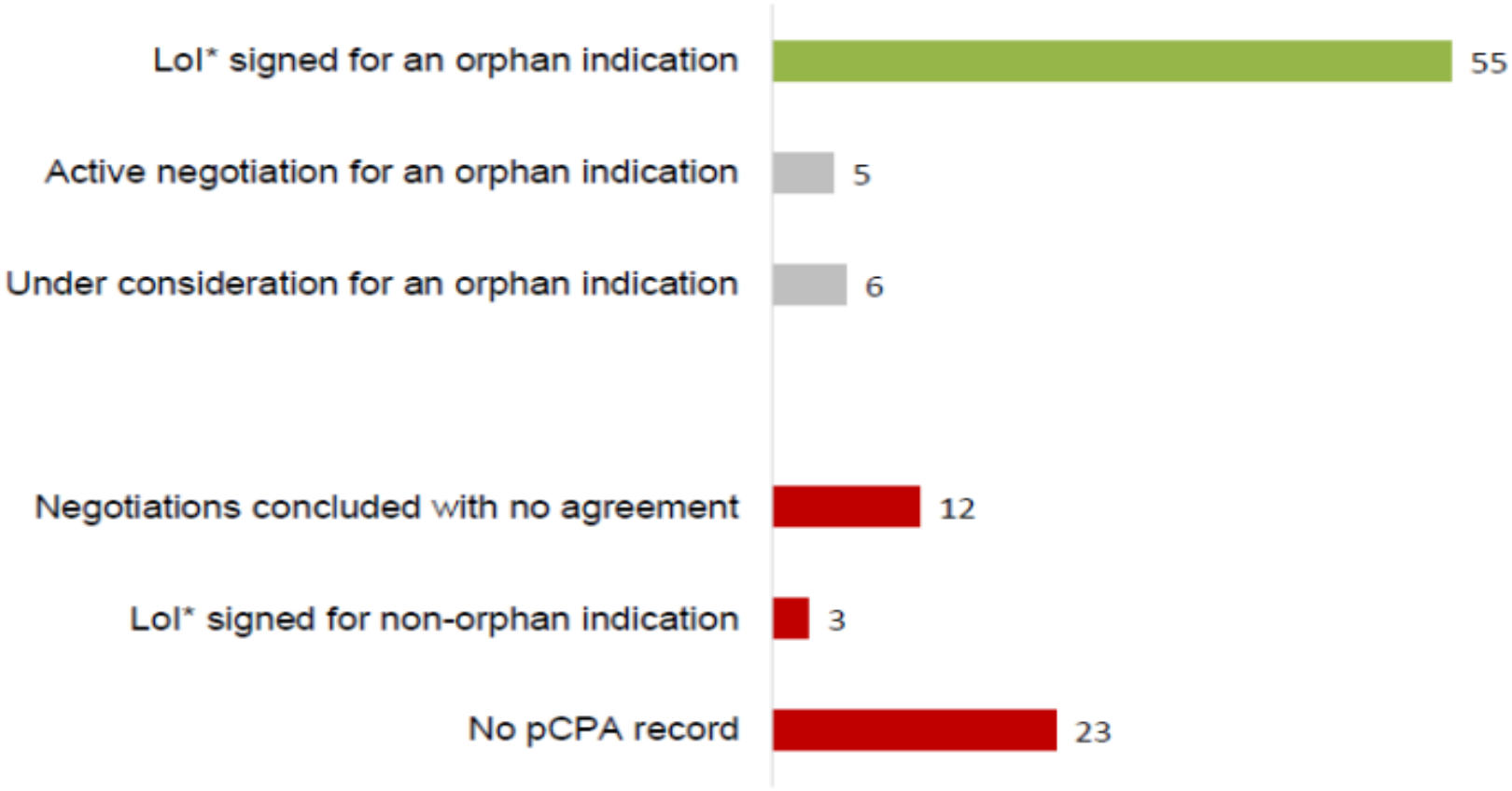
Price negotiations for DRDs can be challenging

The pCPA has completed negotiations relating to an orphan indication for half of the EDRDs in Canada

The pan-Canadian Pharmaceutical Alliance (pCPA) has successfully concluded negotiations with the manufacturers of 55 EDRDs for an orphan indication and is in the process of negotiating or considering negotiations for 11 other EDRDs.

These results point to alignment with the recurrent recommendation from CADTH to reimburse EDRDs conditional on cost-effectiveness being improved.

FIGURE 1.7 pCPA negotiation status for EDRDs, as of June 2021



© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

We may need novel HTA and price negotiation solutions to ensure DRDs can be recommended for funding in a timely manner

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

THANK YOU

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Back up

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Acknowledgements

© 2022 EVERSANA. All Rights Reserved. CONFIDENTIAL.

This analysis can be attributed to all the hard work and support provided by the individuals/organizations listed below:

Authors:

Lindy Forte, EVERSANA

Christine Malmberg, EVERSANA

Kamalpreet Mann, EVERSANA

Gideon Chow, EVERSANA

Richard Lech, EVERSANA

Acknowledgements

Gerry Jeffcott, 3Sixty Public Affairs

William Dempster, 3Sixty Public Affairs

Patrick Mott, EVERSANA

Catherine Hnatiuk, EVERSANA

Ashley Concessio, EVERSANA

William Jeffery, EVERSANA

Sydney Jopling, EVERSANA

Rimsha Chattha, EVERSANA

RAREi

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Limitations

Potential for incomplete data

- Projections were largely based on claims data for historical DRDs; it is possible that this data was incomplete.
- Although FOI requests were made to mitigate this risk, the resulting data may not capture all public spending.

Potential for errors due to linear trending of historical expenditures

- Historical DRDs expenditure was trended in a linear manner and would not capture significant shifts in public spending due to reasons such as inflation, competition, disease awareness, and patent expiration.

Incremental costs were not calculated

- Healthcare spending that could be avoided with access to DRD treatment was not considered in this analysis

Net prices were not considered

- List prices were used throughout this analysis; no assumption for confidential rebates were used.

Abbreviations: DRD = drug for rare disease; FOI = freedom of information.

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

1. Background – increasing development and total cost of DRDs
2. An analysis quantifying the total expected cost of DRDs in Canada
3. Perspective on the cost of DRDs relative to other expenditures
4. Room for improvement in Canada?

EVERSANA®

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.

Background

© 2023 EVERSANA. All Rights Reserved. CONFIDENTIAL.