Patented Medicine Prices Review Board

Conseil d'examen du prix des médicaments brevetés

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June 23, 2020

Elena Lungu, Manager Policy Development

Canada

Patented Medicine Prices Review Board

Conseil d'examen du prix des médicaments brevetés

Insight into the spending on expensive drugs for rare diseases

PMPRB Research Webinar

June 23, 2020

Jared Berger, Policy Analyst

Canada

Introduction

Background

- An increasing number of expensive drugs for rare diseases (EDRDs) have been introduced in recent years, fueling drug spending and raising affordability concerns.

Objective

- This presentation discusses the fast emerging market for EDRDs, from insights into how the development phase leads to the launching of orphan drugs, which combined with high-drug prices makes the EDRDs the fastest growing market segment, pushing the limits of affordability.

Methodology

- For the purpose of this study, EDRDs are defined as medicines with at least one orphan designation through either FDA or EMA, and estimated treatment costs exceeding $100,000 per year for non-oncology drugs and $7,500 per 28 days for oncology drugs.
- Data sources: PMPRB, IQVIA's MIDAS® Database, IQVIA Private Pay Direct Drug Plan Databases, GlobalData Healthcare databases, pCPA, CADTH, Health Canada.

Key Findings

1. Orphan medicines are increasingly dominating the new drug landscape, and with them, there is a rapid increase in the number of EDRDs being introduced

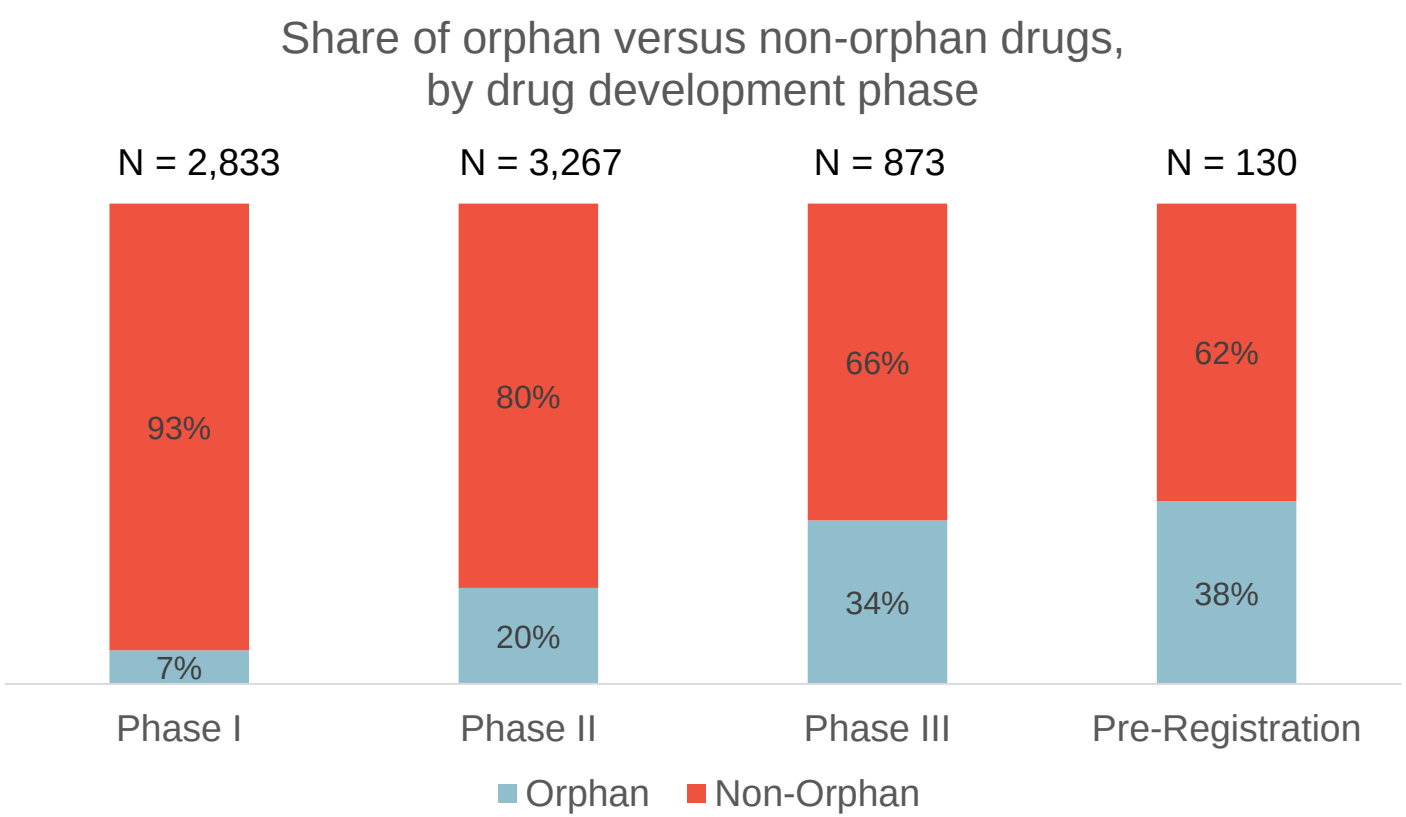
2. EDRDs are the fastest growing market segment in Canada, and are now accounting for nearly one-tenth of pharmaceutical sales, above the OECD norm

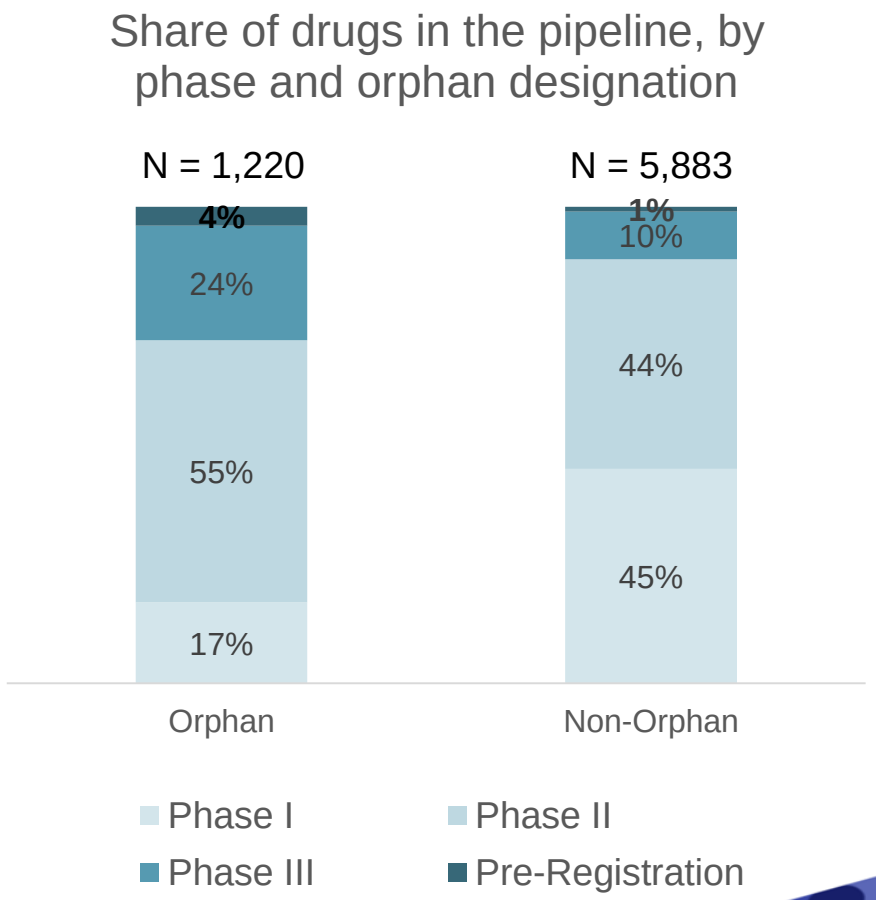
3. Price and affordability of EDRDs are a global concern, especially given that most offer limited or unclear therapeutic benefit, and are not cost-effective at their list price

4. Despite treating small populations, EDRDs have the potential to generate higher sales compared to lower-cost, high-volume drugs

1 Greater share of orphan drugs in the later stages of clinical development

Over one-third of the drugs in Phase III or Pre-registration are orphan, while one-tenth of them are in Phase I
Orphan drugs in the pipeline are more concentrated in Phase III and Pre-registration (28%) than non-orphan (10%) drugs

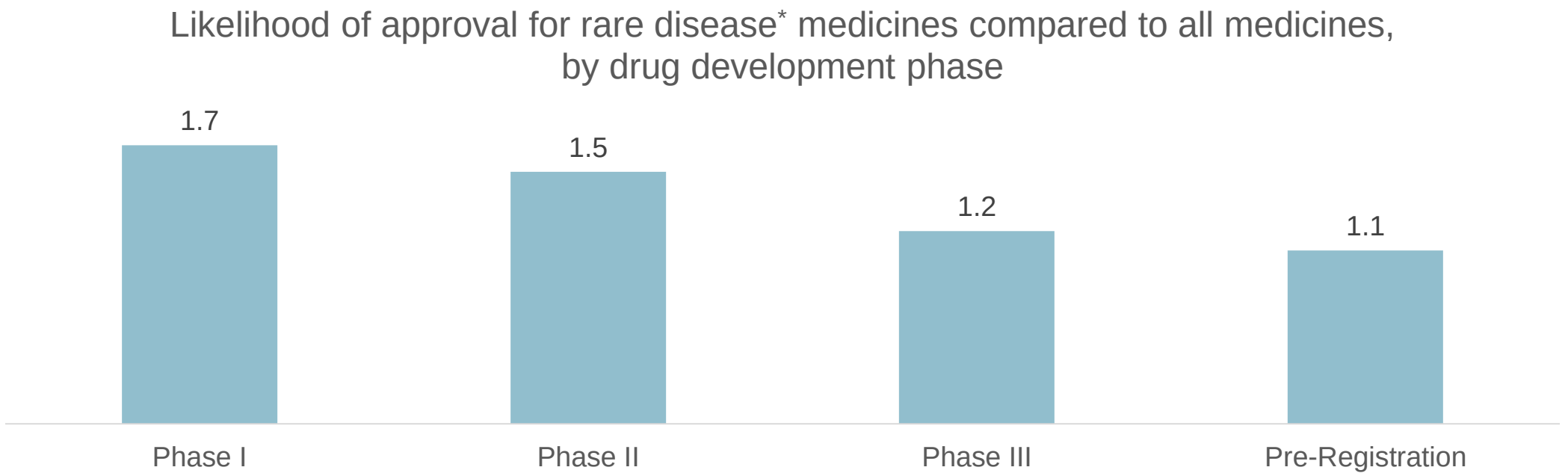




Note: The figures include all pipeline drugs with a highest development stage of phase 1 to pre-registration that are being developed for the Canadian, American, or European Markets. Orphan drugs were defined as a pipeline drug that has been granted an orphan designation in the United States or the EU.
Data source: GlobalData, 2020 (as of May 2020)

1 Drugs for rare diseases are more likely to be approved

The likelihood for drugs for rare disease to be approved is higher at any stake of development, and it is the highest in Phase 1, when these drugs are 1.7 times more likely to be approved compared to all drugs



Notes: GlobalData's 'Likelihood of Approval' database was used to determine the likelihood of approval of medicines to treat rare diseases compared to all diseases. The analysis is restricted to drugs that are approved in the US, the developer has specified the US as an intended market for approval or the manufacturer has not specified any country as the intended market for approval.

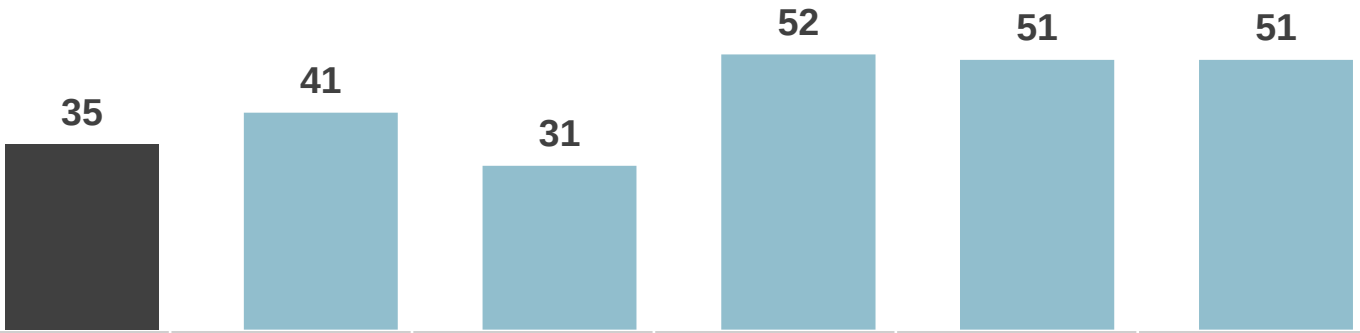
*Rare disease as defined by GlobalData: A rare disease is an indication which is listed by the US FDA and the NIH Genetic and Rare Diseases Information Center (GARD) as rare, and that affects fewer than 200,000 people in the US

Data source: GlobalData, 2020 (extracted on May 28, 2020)

1 Orphan medicines are increasingly dominating the new drug landscape

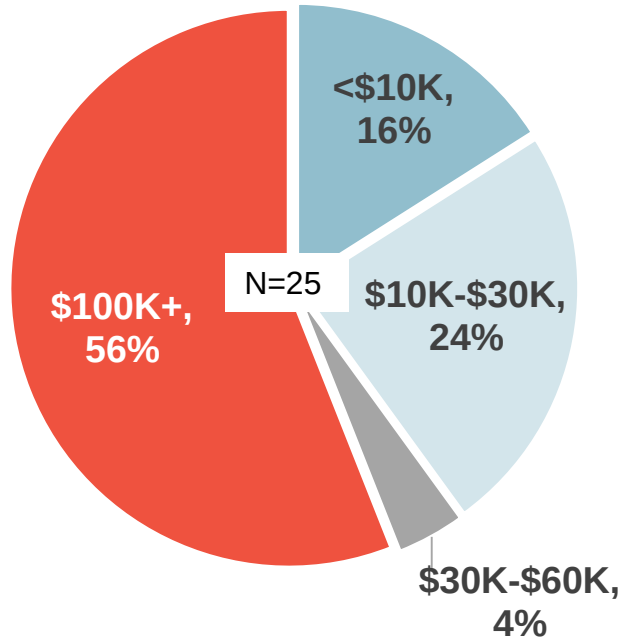
Nearly twice as many orphan drugs (22) were introduced in recent years compared to past averages. Preliminary results suggest that over half of new medicines launched in 2019 cost over $100K

New medicines approved in Canada and the PMPRB7, 2009 to 2019



	2009-2014	2015	2016	2017	2018	2019
Share of orphan medicines	33%	54%	42%	46%	59%	40%
Number of orphan medicines	Average: 12	22	13	24	30	20
		Average: 22				

Share of new medicines by treatment cost, 2019



FDA U.S. FOOD & DRUG ADMINISTRATION

CDER New Drugs Program: 2018 Update

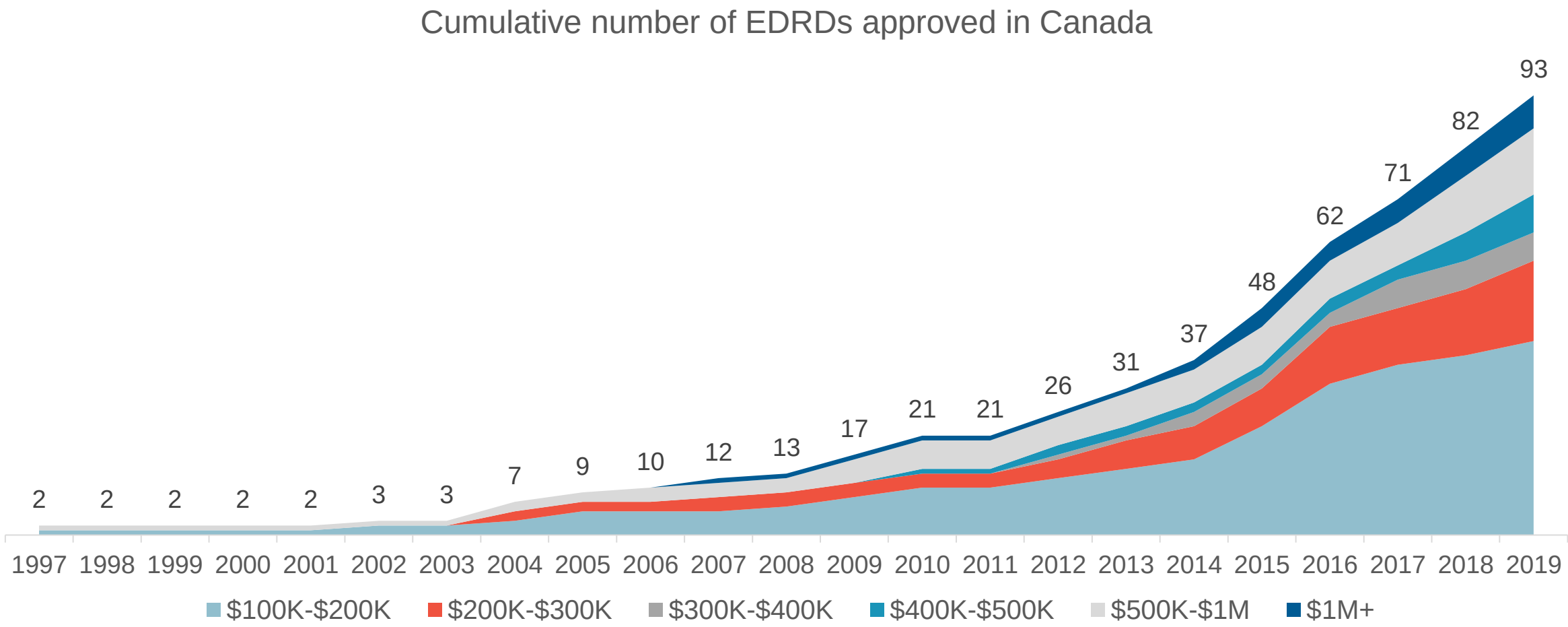
In CY 2018 so far*, CDER has approved 55 NMEs, including 31 orphan drugs

- For the first time ever, the majority of NMEs approved are orphan drugs to treat rare diseases

Data sources: IQVIA MIDAS® Database, 2009 to 2019 (all rights reserved); US Food and Drug Administration, European Medicines Agency, and Health Canada databases; CADTH.

1 Rapid increase in the number of EDRDs being introduced

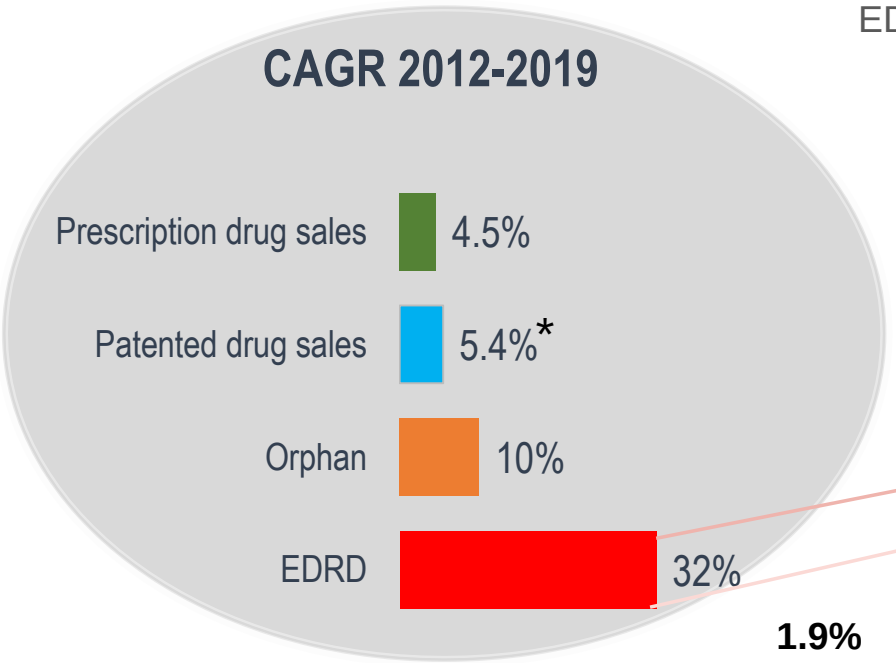
By the end of 2019, 93 EDRDs have been approved for sale in the Canadian market



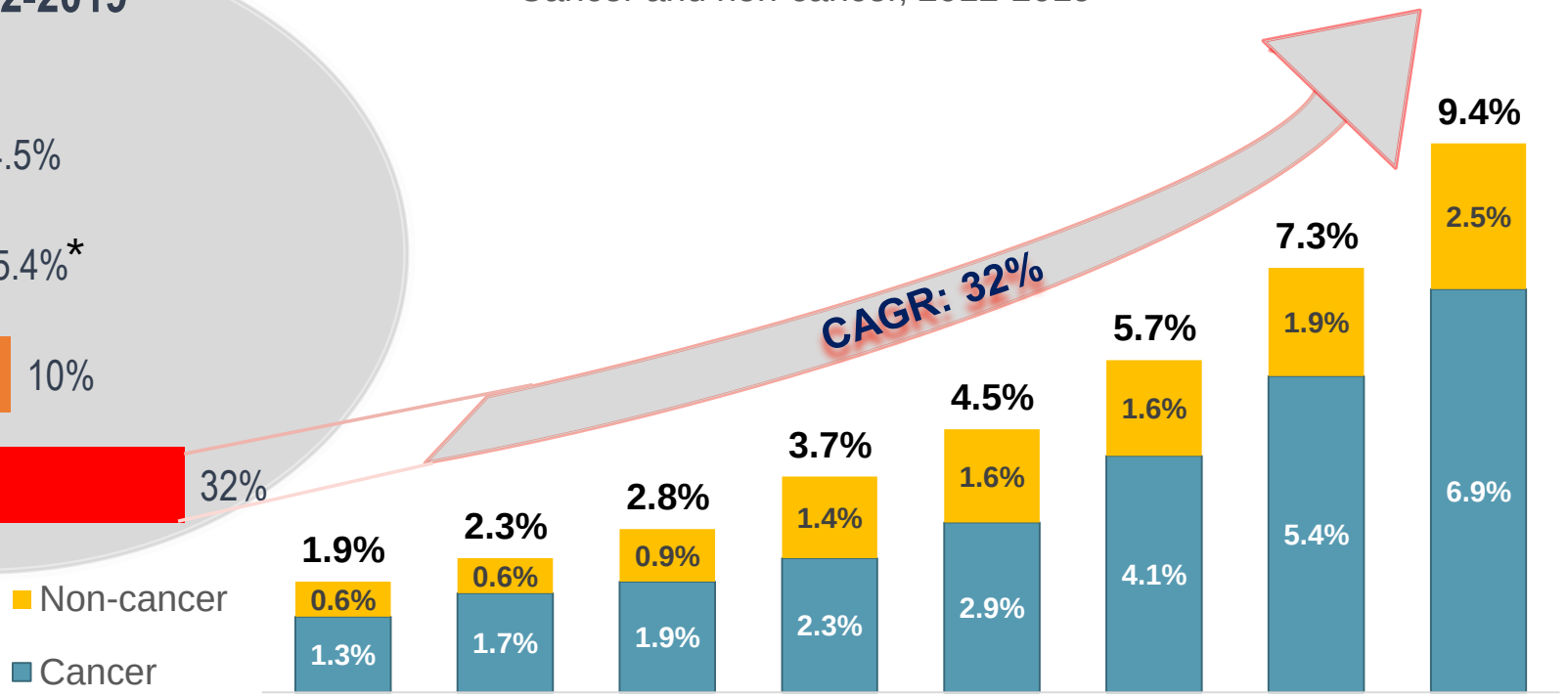
Data source: PMPRB; CADTH; IQVIA MIDAS® Database.

2 EDRDs are the fastest growing market segment

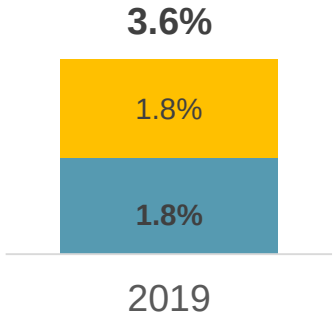
Despite small patient populations, EDRDs now account for nearly one-tenth of the drug sales



EDRD share of the pharmaceutical sales in Canada, Cancer and non-cancer, 2012-2019



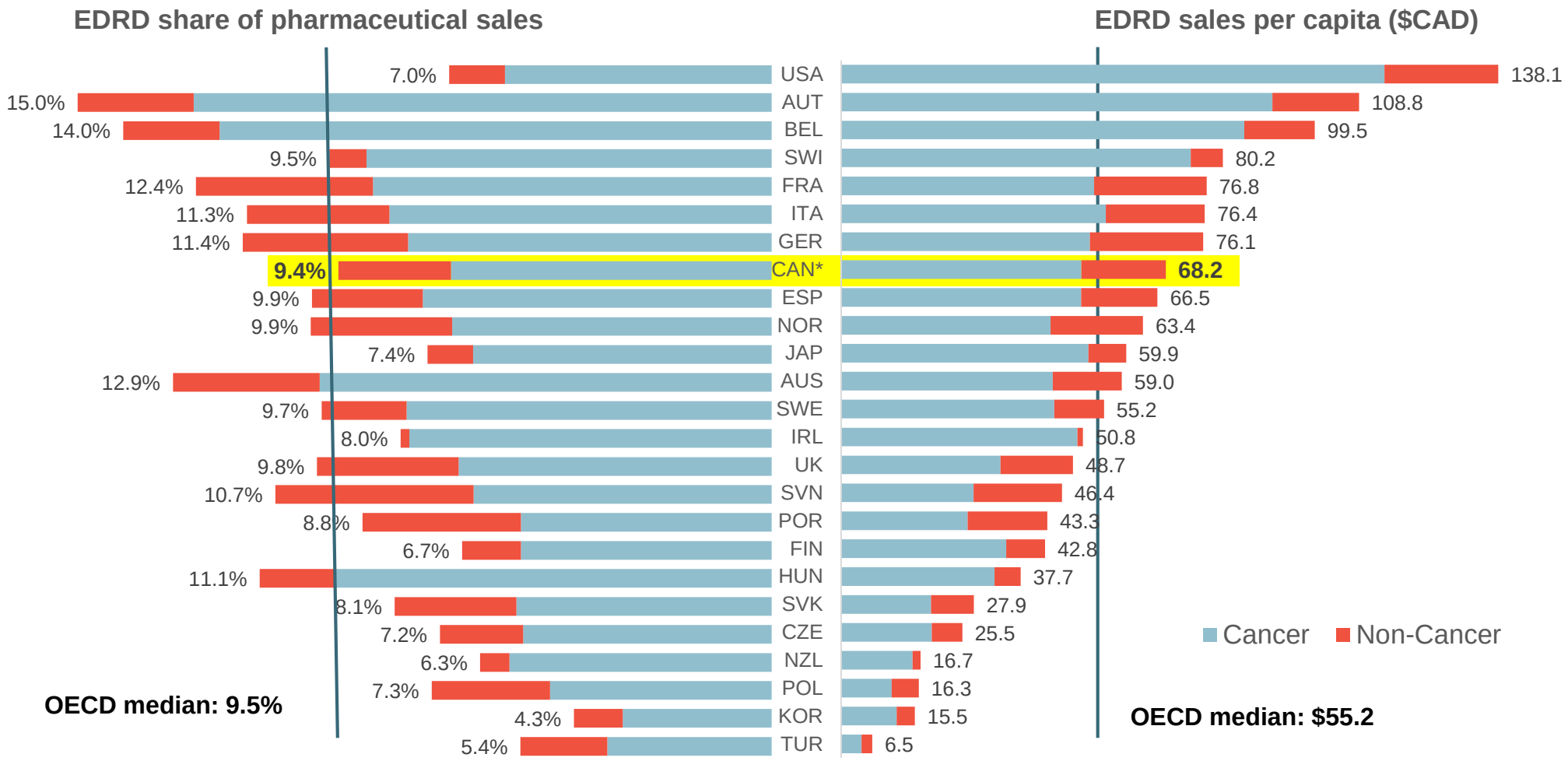
EDRD share of drug costs in private plans, 2019



	2012	2013	2014	2015	2016	2017	2018	2019
Cumulative number of EDRDs approved	26	31	37	48	62	71	82	**93**
Sales per capita	$11	$13	$16	$23	$29	$38	$50	**$68**

Note: *The patented drug sales growth rate is reported from 2012 to 2017.
Data source(s): PMPRB, IQVIA MIDAS® Database, 2012 to 2019 (all rights reserved), IQVIA Private Pay Direct Drug Plan Databases.

2 EDRD spending in Canada is above the OECD norm

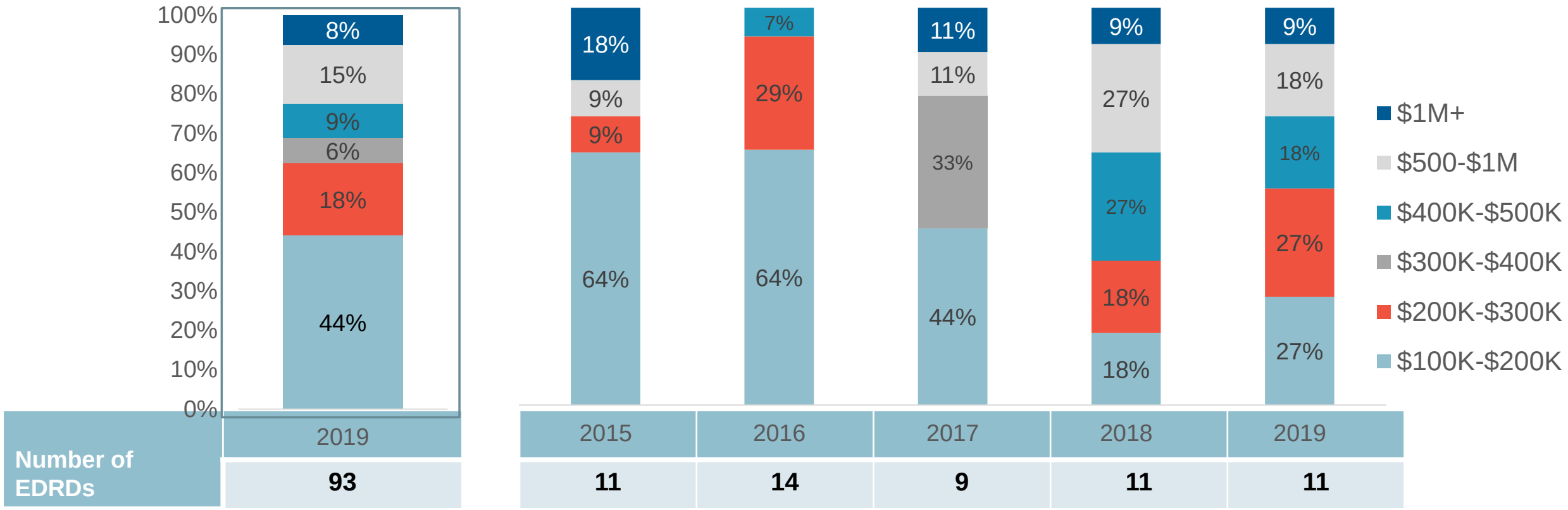


Note: OECD countries lacking hospital sales data were excluded from the analysis.
* Estimated by complementing MIDAS™ with PMPRB, and IQVIA Private Pay Direct Drug Plan Databases Data
Data source(s): PMPRB, IQVIA MIDAS® Database, 2019 (all rights reserved).

3 The escalating treatment cost of EDRD drugs is a concern

Over half (56%) of all EDRDs available in Canada cost over $200K annually, and recent years have seen even greater shares (82% in 2018 and 73% in 2019)

Share of EDRDs approved in Canada by treatment cost, as of 2019 and by year of approval

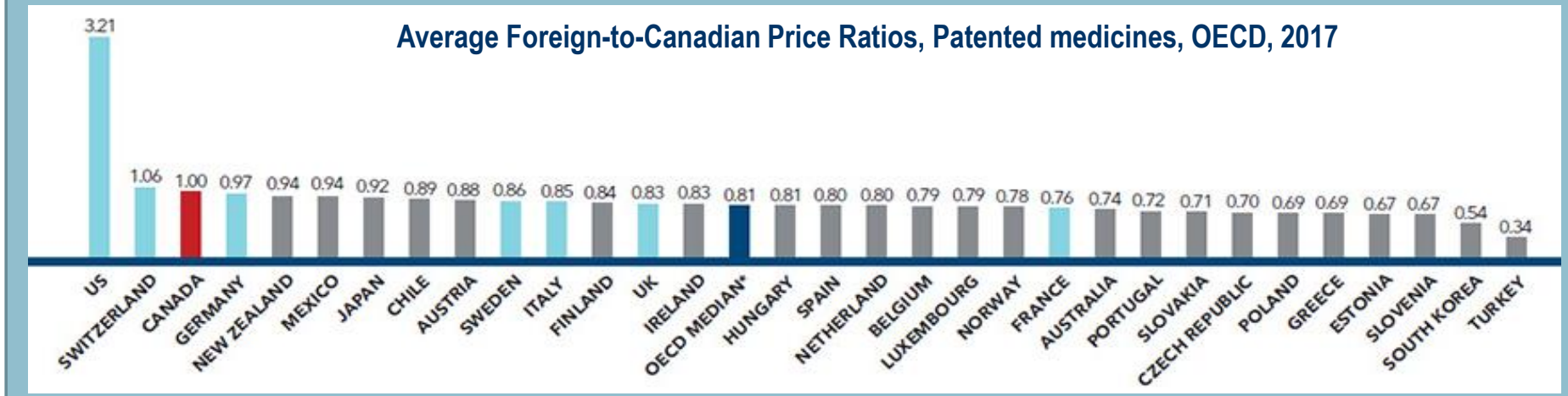


	2019	2015	2016	2017	2018	2019
Number of EDRDs	93	11	14	9	11	11

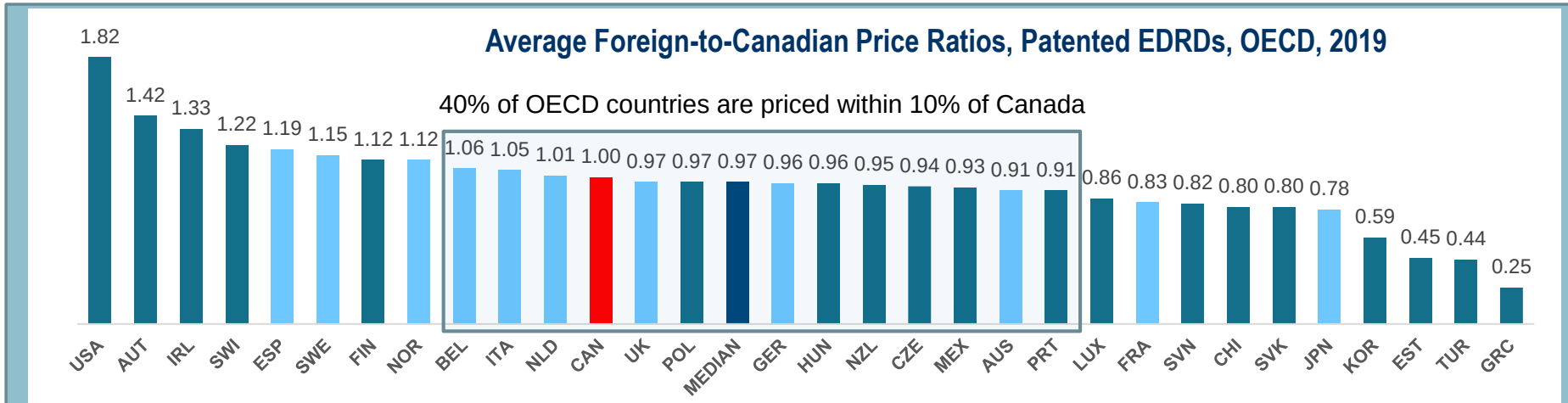
Data source: PMPRB; CADTH; IQVIA MIDAS®

3 EDRD pricing is an international concern, as all countries pay high prices

EDRD list price is just a sticker price, with closer international alignment than the prices of patented medicines in general



Canadian prices are **3rd** highest



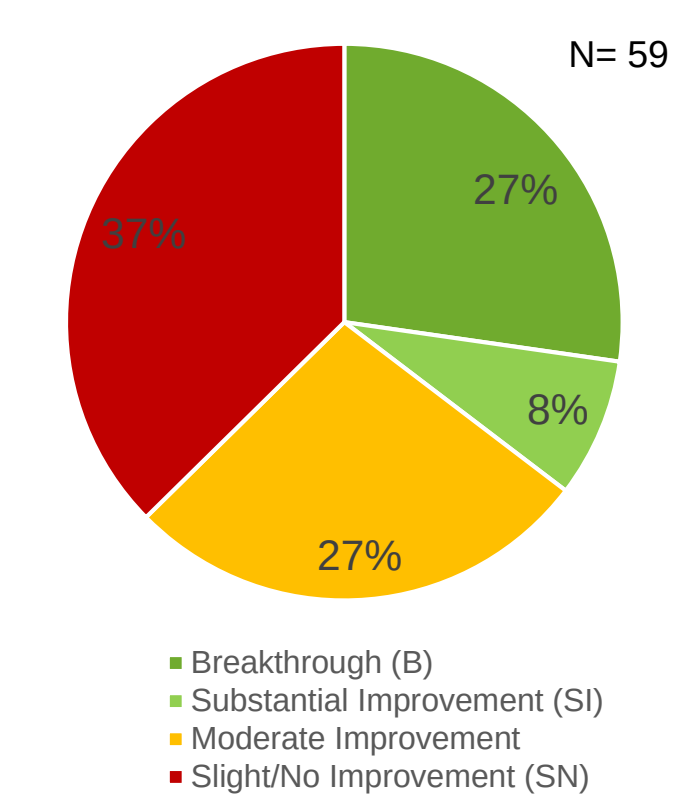
Price for rare disease drugs are more aligned, as rebated prices are negotiated confidentially and individually by countries

Data source PMPRB, IQVIA MIDAS® Database, 2019 (all rights reserved).

3 Most EDRDs offer limited or unclear therapeutic benefit, and are not cost-effective at their list price

Many bring moderate, slight or no therapeutic improvement over comparators, and would require a price reduction greater than 80% to achieve an ICER of $100K

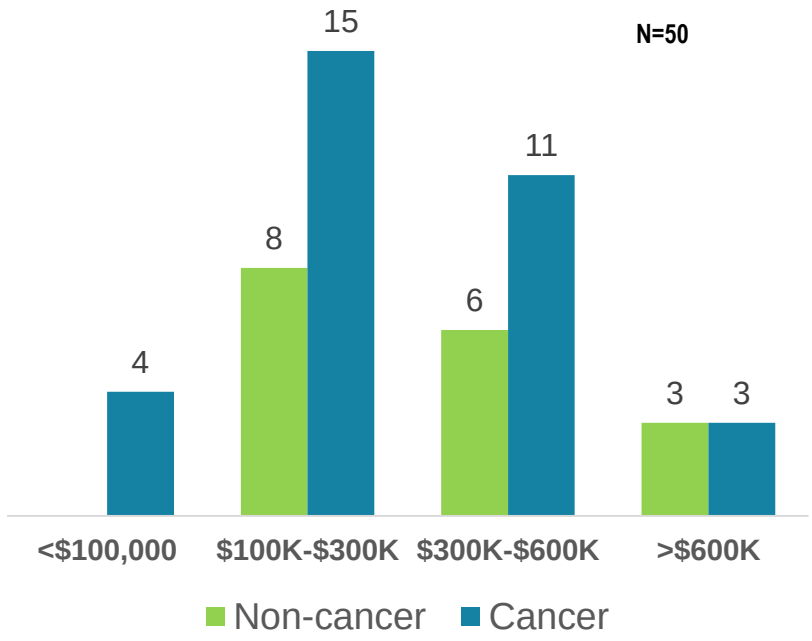
Level of Therapeutic Improvement, PMPRB



Trade Name (EDRD)	Indications	Price reduction for $100K/QALY
Orkambi (lumacaftor/ivacaftor)	Cystic Fibrosis (Ages 6+)	97%
Strensiq (asfotase alfa)	Hypophosphatasia , pediatric-onset	96%
Ilaris (canakinumab)	Systemic juvenile idiopathic arthritis	86%-90%
Procysbi (cysteamine)	Nephropathic cystinosis	95%
Vimizim (elosulfase alfa)	Mucopolysacchari -dosis IVA	97%
Kalydeco (ivacaftor)	Cystic Fibrosis	98%
Spinraza (Nusinersen)	Spinal Muscular Atrophy	>95%
Kuvan (sapropterin dihydrochloride)	Phenylketonuria	82%
Kanuma (sebelipase alfa)	Lysosomal acid lipase deficiency	>95%
Revestive (teduglutide)	Short Bowel Syndrome (SBS)	80%
Radicava (edaravone)	amyotrophic lateral sclerosis	>95%

HTA – CADTH

Distribution of cancer and non-cancer EDRDs by cost-effectiveness ratios



Data source: CADTH, PMPRB (April 2020)

14

3 Affordability of EDRD is a concern in Canada

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Blood-disorder drug Soliris to receive limited PharmaCare coverage in B.C.

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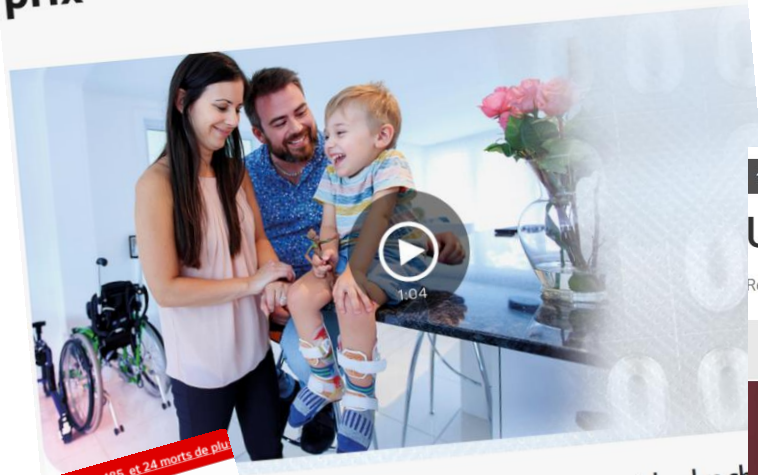
La loterie des médicaments hors de prix



n de Repentigny, a reçu le traitement le plus ch
rapie génique vendue près de 3 millions de
lus de traitements coûtent une fortune et le

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published: 2019-08-29
received: 2019-08-29 03:03 (EST)

THE GLOBE AND MAIL

Globe and Mail (Ontario)
News | A1, Words: 1,055

'Nobody can afford this'

Olivia Little's parents wonder how they will treat her rare disease after a drug maker tweaked the formula for eye drops, which raised the price from a few thousand dollars a year to $103,272

by: Kelly Grant

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CBC News -
Posted: Apr 08, 2019 4:57 PM ET | Last Updated: April 8

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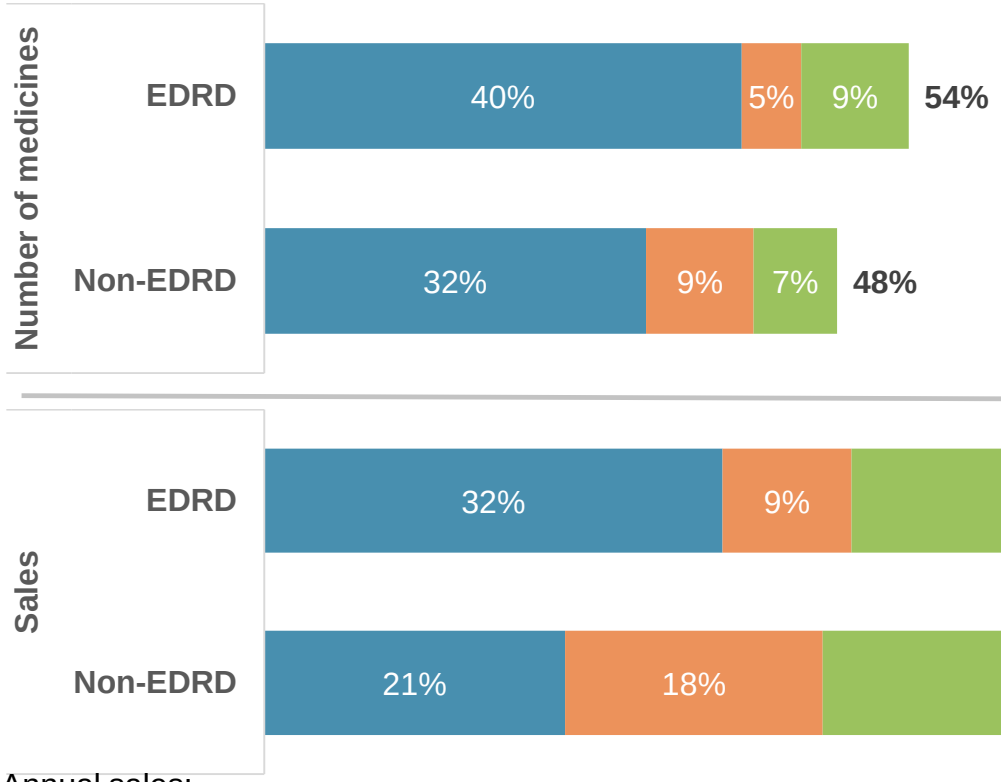
Valérie Fortin | TVA Nouvelles | **Publié le 4 décembre 2019 à 22:19** - Mis à jour le 4 décembre 2019 à 22:24

3 Canada is taking steps to improve drugs access and affordability

- The federal government amended the Patented Medicine Regulations, giving the PMPRB the tools and information it needs to protect Canadian consumers from excessive pricing
- To help Canadians with rare diseases, Budget 2019 proposes to invest up to $1 billion over two years, starting in 2022–23, with up to $500 million per year ongoing
- Health Canada's Priority Review status assigns eligible submissions a shortened review target of 180 days, in comparison to 300 days for nonpriority. Priority Review status may be granted to drug submissions intended for the treatment, prevention or diagnosis of serious, life-threatening or severely debilitating illnesses or conditions where a) there is no existing drug on the Canadian market with the same profile or b) where the new product represents a significant improvement in the benefit/risk profile over existing products.
- Market authorization under the Notice of Compliance with Conditions (NOC/c) Policy allows Health Canada to provide earlier market access to potentially life-saving drugs. Conditions associated with market authorization allow Health Canada to monitor the drug through enhanced post-market surveillance.

4 EDRDs may generate higher sales than lower-cost, high-volume drugs

EDRD and non-EDRD distribution of medicines and of sales,
by the highest annual sales in the first three years after launch in Canada



	EDRD	Non-EDRD
Average Sales	$28M	$34M
Median Sales	$10.1M	$9.7M

Medicines for orphan diseases, despite smaller patient populations, have the commercial potential to generate revenue for the originator companies at least as great as for non-orphan medicines

Note: Includes medicines launched in Canada since 2005; EDRDs had at least 2 years of sales.
Sample size: EDRDs; *N* = 43; Non-EDRDs; *N* = 255.
Data source: IQVIA MIDAS® Database (all rights reserved); PMPRB, US Food and Drug Administration, European Medicines Agency, and Health Canada databases.

Patented Medicine Prices Review Board

Conseil d'examen du prix des médicaments brevetés

Insights into the market size of patented medicines in Canada

PMPRB Research Webinar

June 23, 2020

Jihong Yang, Policy Analyst

Canada

Introduction

- Important amendments have been brought to the Patented Medicines Regulations and the PMPRB has been consulting on its Guidelines that will implement these amendments.
- The amended Regulations require the Board to consider the Market Size factor in its determination of excessive price. This will ensure that the impact of paying for the medicine for everyone who needs it is taken into account in determining if its price is excessive.
- The market size is a function of both price and volume; the larger the size of the market for the medicine in Canada, the greater the impact of its price.
- In the Backgrounder document accompanying the draft Guidelines, the PMPRB has estimated that a $12 million annual market size for a new medicine would result in new spending on patented medicines commensurate with the annual growth in GDP in Canada.
- This study examines the market size for patented medicines launched over the last 20 years in Canada, to inform the policy decision around the application of the market size regulatory factor.

Average annual patented medicines sales, 2014 to 2018 ($B)	**$14.9 B**
Average annual growth in patented medicine sales, in line with the growth rate in GDP	**$432 MB**
Average number of new patented medicines introduced per year, 2014 to 2018	**36**
Average annual affordability threshold per medicine	**$12M**

Methods

- All patented medicines introduced from 1998 to 2018 were identified along with their annual sales. Patented medicines are examined at the molecule level.
- The results are based on “real sales”, determined by converting all historical sales into 2018 dollars using CPI rates, as reported by Statistics Canada
- The sales reported are not reflective of confidential discounts given to payers
- Medicines with 3 years of sales post introduction were identified and their maximum annual sales in any of those years were determined: N=639
- Similarly, medicines with 10 years of sales post introduction were identified and their maximum annual sales in any of those years were determined: N=338
- Treatment costs was calculated for EDRDs using CADTH reports if available; otherwise higher of the annual average treatment cost from private or public drug plan data
- Data sources: PMPRB, IQVIA MIDAS, IQVIA Private Drug Plan Database, NPDUIS Public Drug Plan Database, Health Canada

Key Findings

1. Historically, a minority of medicines realized relatively high sales over their life time, however they make up the majority of overall patented medicines sales

2. Medicines launched in recent years are more likely to reach higher sales than earlier launches, and this is mainly driven by higher-cost drugs

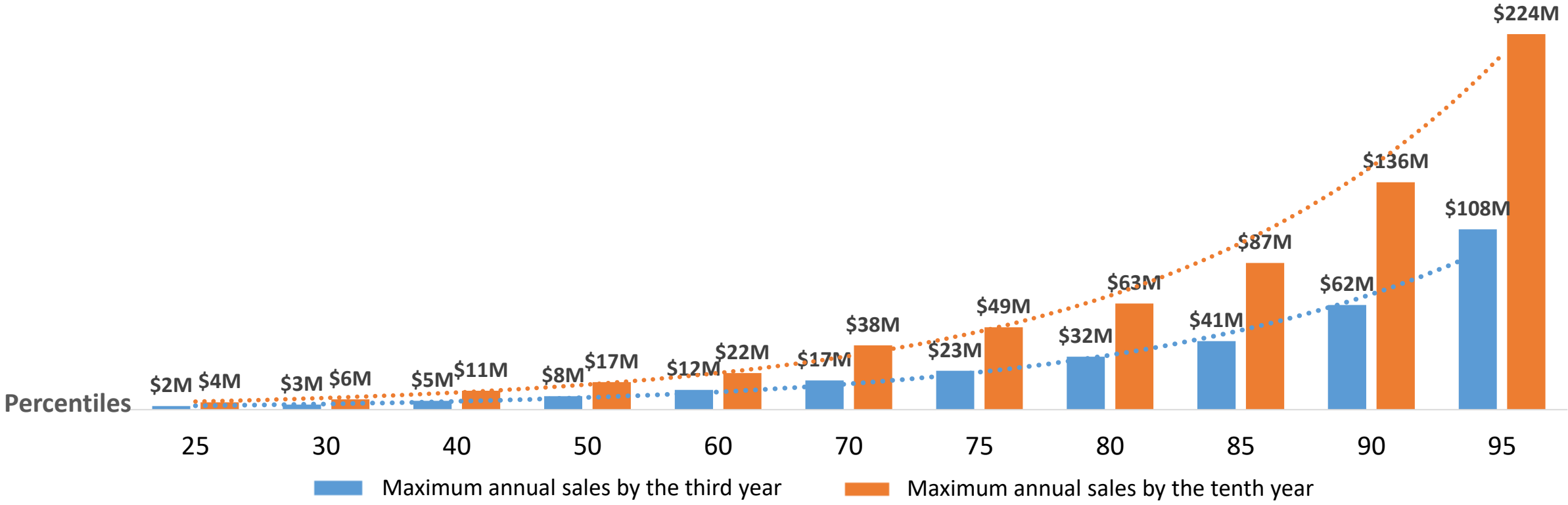
3. Patented drug sales are increasingly concentrated in higher-sales medicine but also on higher-cost medicines

4. Higher-cost medicines are more likely to result in higher-sales

1 A minority of the medicines realize relatively high sales over their life time

25% of the medicines realize over $23M and $49M in annual sales by the third year and tenth year, respectively

Percentiles of the maximum annual sales by the third year and by the tenth year for the patented medicines in Canada*

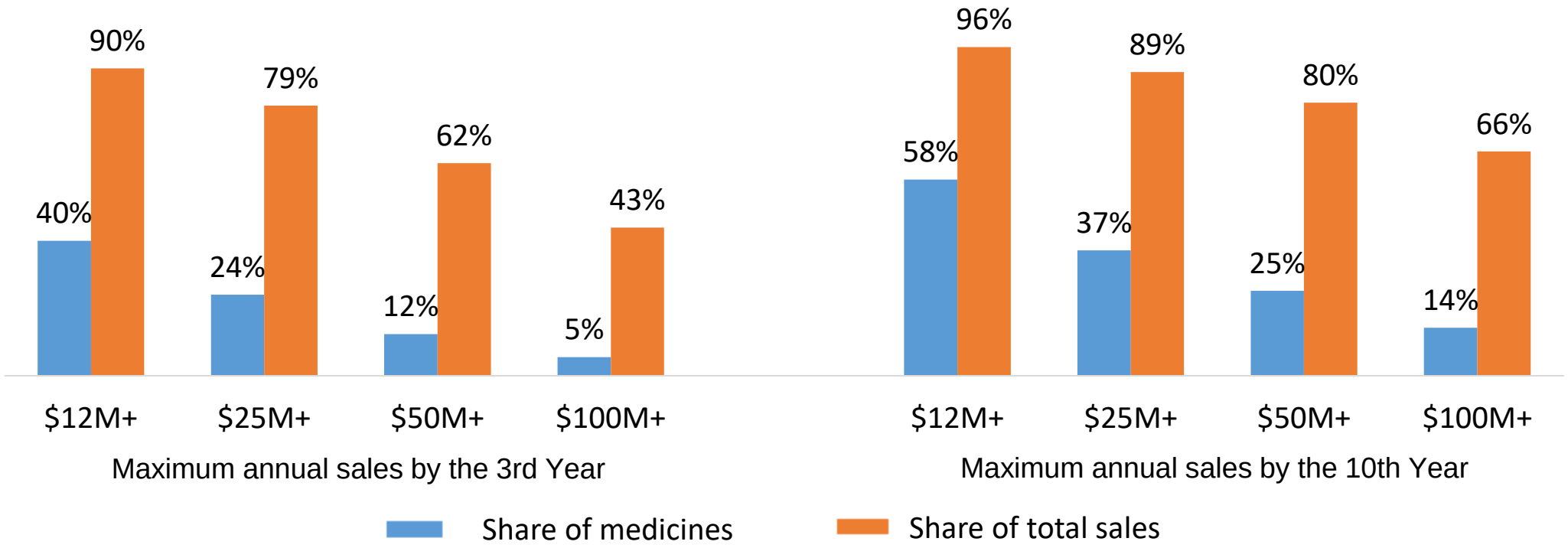


*Included patented medicines launched after 1998 in Canada; Sample Size: N=639 for the max annual sales by the 3rd year: ; N=338 for the max annual sales by the 10th year
Data source: PMPRB, 2018; CPI applied to bring historical sales into 2018 value

1 Medicines that realize relatively high annual sales account for the majority of overall patented medicines sales

37% of the medicines exceed $25M in annual sales over the first 10 years after introduction, however, these medicines account for the 89% of the total maximum annual sales for all medicines

Patented medicines in Canada, share of medicines and share of sales, by annual sales thresholds, by the 3rd and 10th year after introduction*

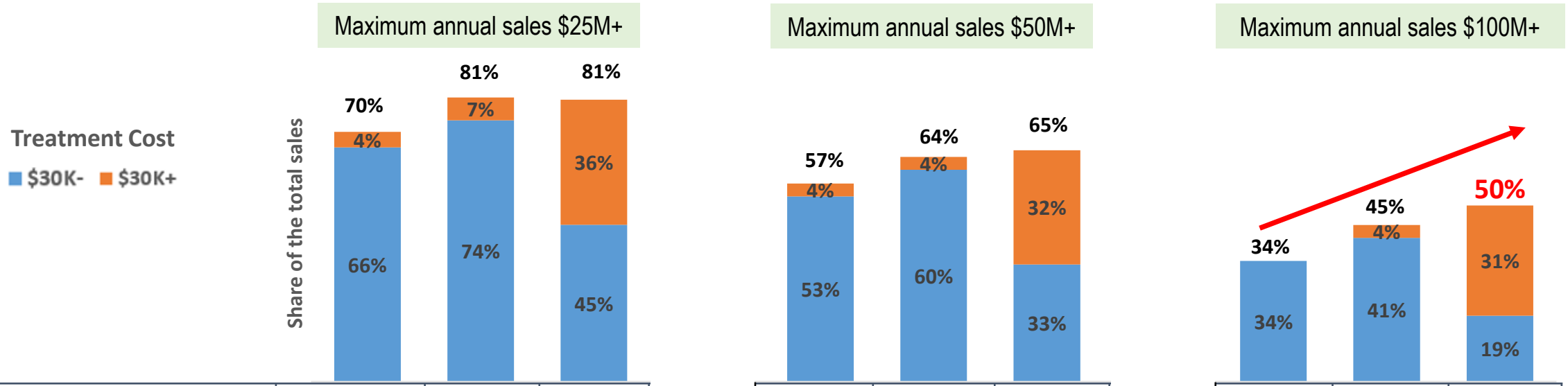


*Included patented medicines launched after 1998 in Canada; Sample Size: N=639 by the 3rd year: ; N=338 by the 10th year
Data source: PMPRB, 2018; CPI applied to bring historical sales into 2018 value

2 Newer medicines are increasingly realizing higher sales

7% of meds launched during 2011-2015 realized over $100M in sales by their 3rd year, and make up 50% of the patented sales in their cohort

Patented medicines in Canada, share of sales and share of medicines by the 3rd year after introduction, by annual sales thresholds, treatment cost and launch year cohort *



Launch year cohort / Share of total meds	2001-2005	2006-2010	2011-2015
$30K+	2%	3%	6%
$30K-	18%	19%	17%

2001-2005	2006-2010	2011-2015
1%	1%	4%
10%	11%	8%

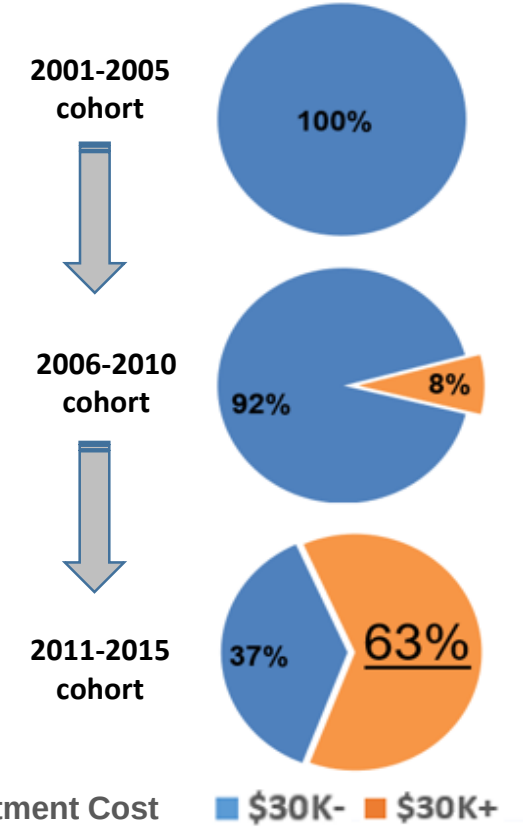
2001-2005	2006-2010	2011-2015
0%	1%	**3%**
4%	5%	**4%**

*Included patented drugs launched after 2001 in Canada; Sample Size: 2001-2005 cohort N=164; 2006-2010 cohort N=155; 2011-2015 cohort N=189
Data source: PMPRB, 2018; CPI applied to bring historical sales into 2018 value

2 The high-sale market segment is mainly driven in recent years by higher-cost drugs

Higher-cost medicines account for 63% of the sales for medicines with $100M+ in annual sales by the third year (2011-2015 cohort), compared to 8% five years ago

Low-cost and high-cost medicines' contribution to the total annual sales of over $100M by the third year, by launch year cohort *



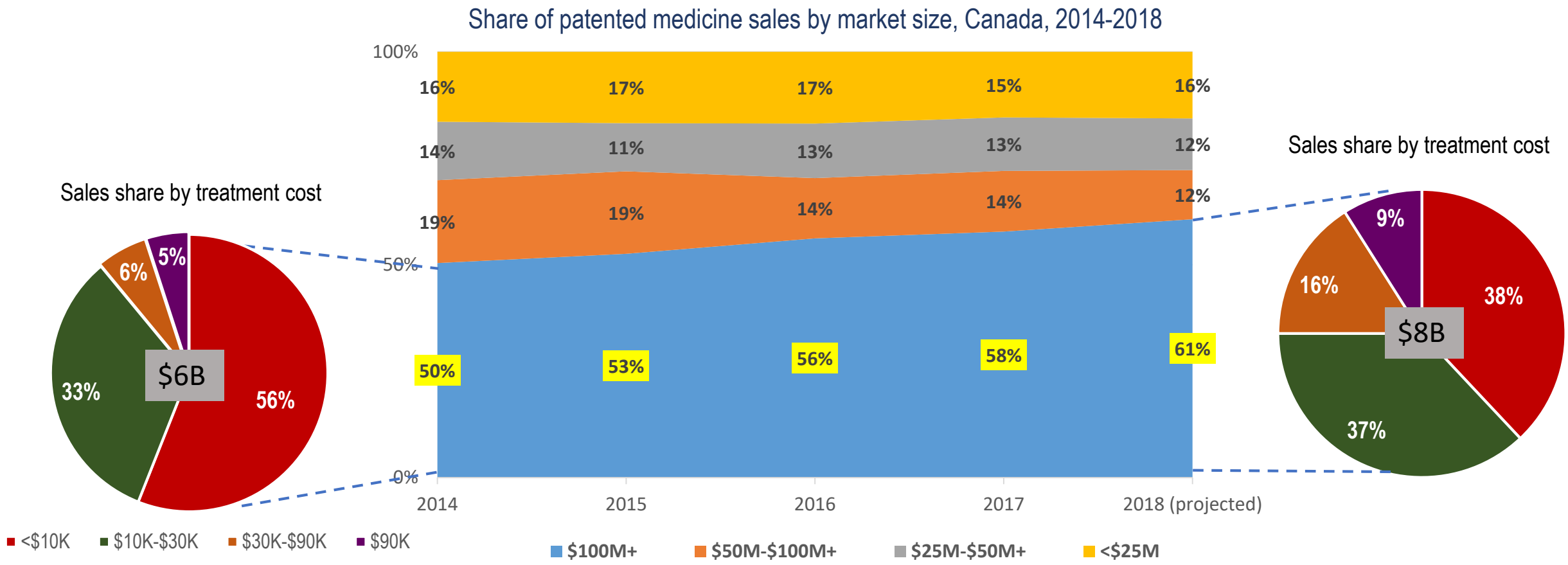
Highest selling medicines, 2019

Medicine (Brand)	Therapy	2019 Sales (IQVIA MIDAS®)	Treatment Cost**
Infliximab (Remicade) B, O	Arthritis	$1,185M	$30,771
Adalimumab (Humira) B, O	Anti-TNF	$854M	$17,778
Aflibercept (Eylea) O	Oncology	$553M	$9,394
Sofosbuvir:Velpatasvir (Epclusa)	Hepatitis C	$497M	$56,666
Ustekinumab (Stelara) B	Psoriasis	$413M	$22,435
Ranibizumab (Lucentis) B, O	AMD	$324M	$9,902
Metformin:Sitagliptin (Janumet)	Diabetes	$300M	$956
Etanercept (Enbrel) B, O	Anti-TNF	$283M	$16,934
Apixaban (Eliquis)	Hypertension	$292M	$906
Ibrutinib (Imbruvica) B, O, EDRD	Oncology	$275M	$7,615 per 28 days
Pembrolizumab (Keytruda) B, O, EDRD	Oncology	$274M	$11,733 per 28 days

*Included patented drugs launched after 2001 in Canada; Sample Size: 2001-2005 cohort N=164; 2006-2010 cohort N=155; 2011-2015 cohort N=189
Data source: PMPRB, 2018; CPI applied to bring historical sales into 2018 value; B, O Biologic, Orphan
** Data source: PMPRB NUDUIS databases and IQVIA Private Pay Direct Drug Plan Databases for non-EDRDs; CADTH reports for EDRDs

3 Patented drug sales are increasingly concentrated in higher-sales medicines

Medicines with $100M+ in annual sales account for an estimated 61% of patented medicine sales in 2018, compared to only 50% in 2014, with a CAGR of 10%



Data source: PMPRB, 2017; Sales 2018 was projected; CPI applied to bring historical sales into 2018 value

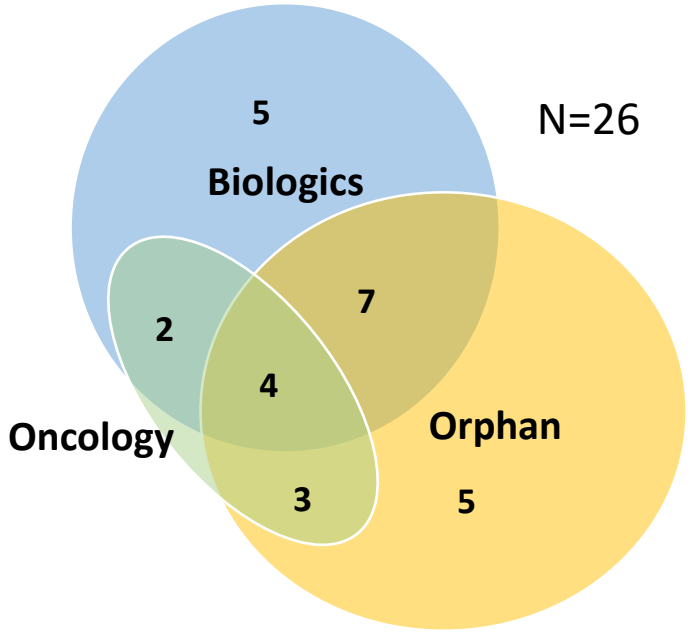
3 Increasing number of higher-cost medicines contributes to very high sales

8 medicines with treatment cost over $90K expected to realize sales over $100M in 2019, compared to only 2 in 2014

Number of medicines realizing $100M+ sales by treatment cost

Treatment Cost	2001	2010	2014	2017	2019 (Forecasted)
$10K-$30K	1	5	7	11	14
$30K-$60K	-	2	1	3	4
$60K-$90K	-	-	1	1	1
$90K+	-	2	2	5	8

Number of specialty medicines with the sales over $100M in 2017*



* In total, there were 41 patented medicines with over $100M sales in 2017

Data source: PMPRB, 2017; Sales 2019 was projected; CPI applied to bring historical sales into 2018 value

4 Higher-cost medicines are more likely to reach high sales than lower-cost

A larger share of medicines with treatment costs exceeding $10K realized over $10 million in sales compared lower treatment costs medicines (<$10K)

Distribution of patented drugs by highest sales* in the first 3 years after launch

Annual Treatment cost	Share of drugs with sales in $10M-$50M	$50M-$100M	$100M+	∑ $10M+ in sales	Share of total sales	Avg. sales per drug
<$10K	33%	6%	5%	44%	91%	$24M
≥$10K	36%	10%	9%	54%	94%	$33M
≥$30K	38%	4%	9%	51%	94%	$33M
$90K+	32%	5%	11%	47%	92%	$28M

*Included patented drugs launched after 2001 in Canada; Sample Size: <$10K: N=275; $10K+: N=129; $30K+: N=79; $90K+: N=38
Data source: PMPRB, 2018; CPI applied to bring historical sales into 2018 value

Patented Medicine Prices Review Board

Conseil d'examen du prix des médicaments brevetés

THANK YOU

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