



Patented  
Medicine Prices  
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Conseil d'examen  
du prix des médicaments  
brevetés

# What is the "Expense" for Expensive Drugs for Rare Diseases?



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~ No conflict of interest to declare ~

Canada 

# Less than 1% of the Canadian population accounts for 42% of patented medicine sales

Share of Sales for High-Cost Patented Medicines, 2006 to 2017

Between 2006 and 2017, the number of medicines in Canada with an annual per beneficiary cost of at least \$10K increased by over 200% and now account for 42% of patented medicine sales.

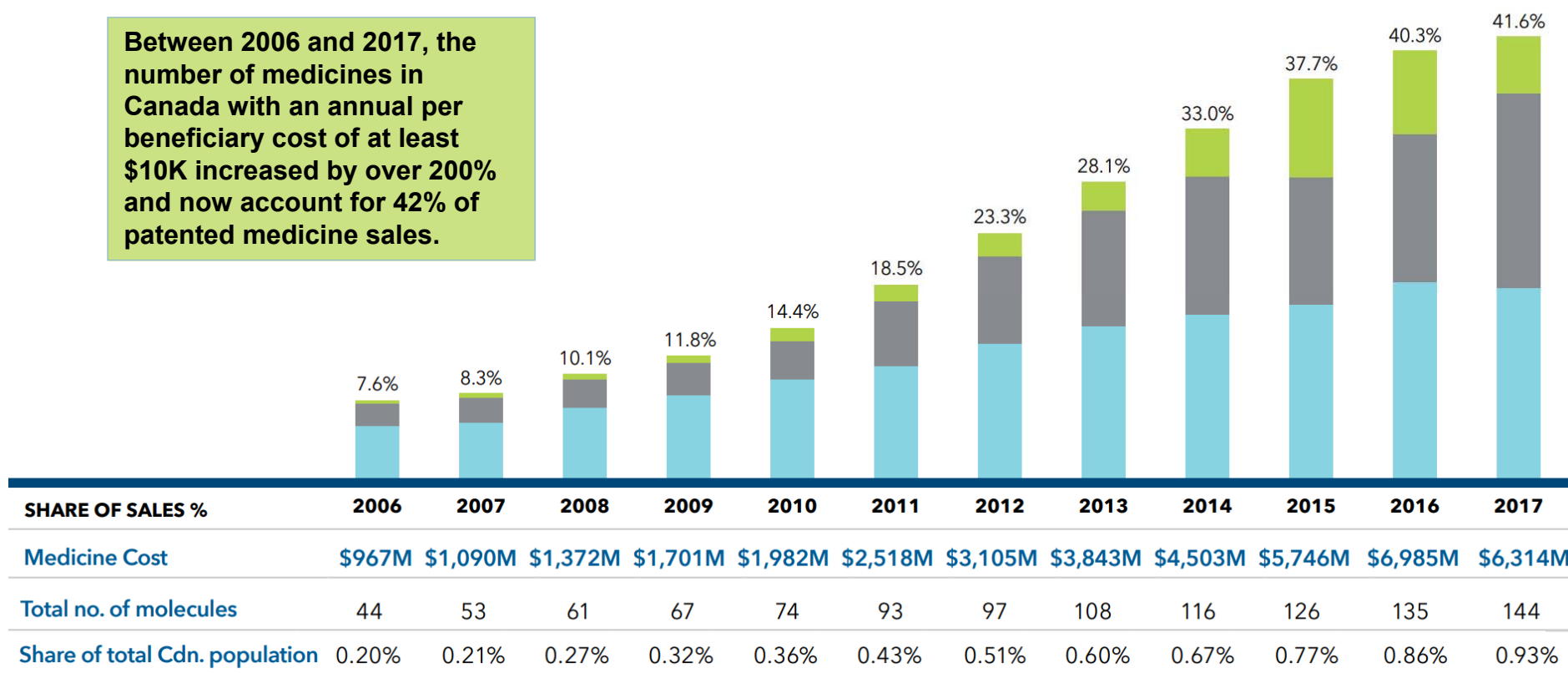


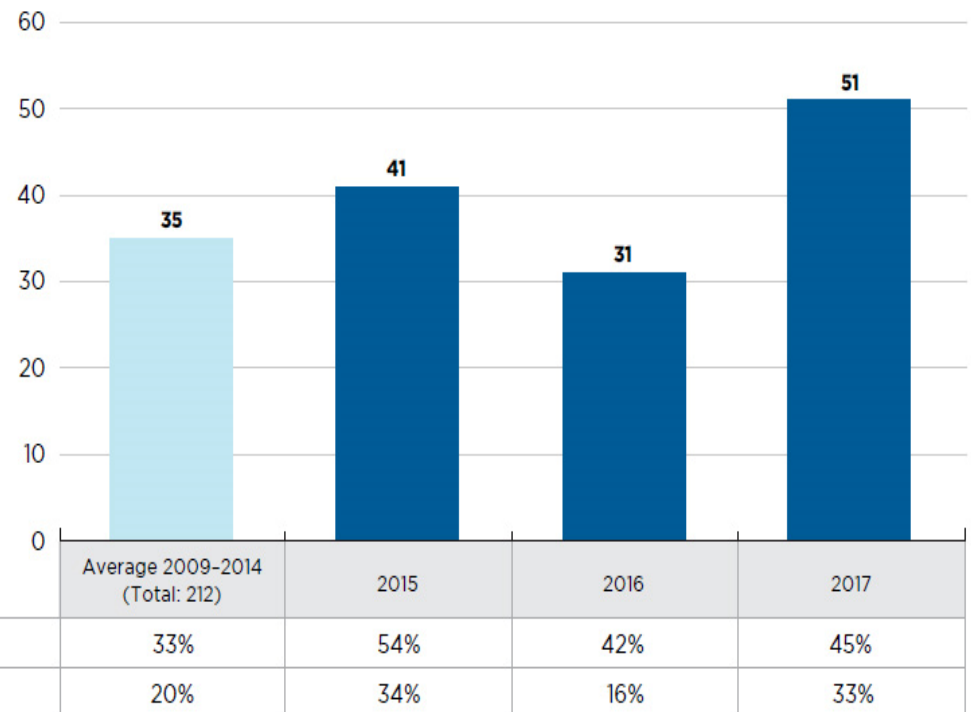
Figure source: PMPRB Annual Report, 2017.

Data source: PMPRB; IQVIA Private Pay Direct Drug Plan Database, 2006–2017.

# Is high-cost becoming the norm for new drug launches?

- Orphan medicines are increasingly dominating the market, accounting for nearly half of new launches
- Over one quarter of the new medicines in 2016 and 2017 were developed for the treatment of cancer and many came with a high cost, averaging approximately \$13,700 for a 28-day treatment
- The majority of non-oncology medicines launched in 2016 and 2017 were high-cost, with 31 of the 37 with available treatment costs exceeding \$10,000 annually

**MEDS ENTRY WATCH 2017**  
New medicines launched in Canada and the PMPRB7, 2009 to 2017



	Average 2009-2014 (Total: 212)	2015	2016	2017
Share of orphan medicines	33%	54%	42%	45%
Share of oncology medicines	20%	34%	16%	33%



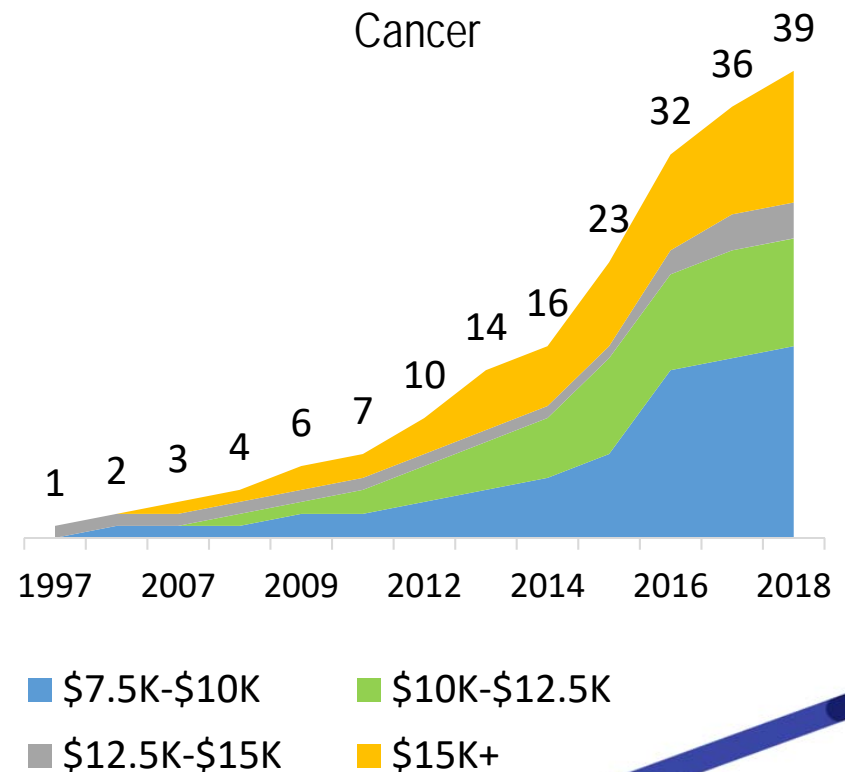
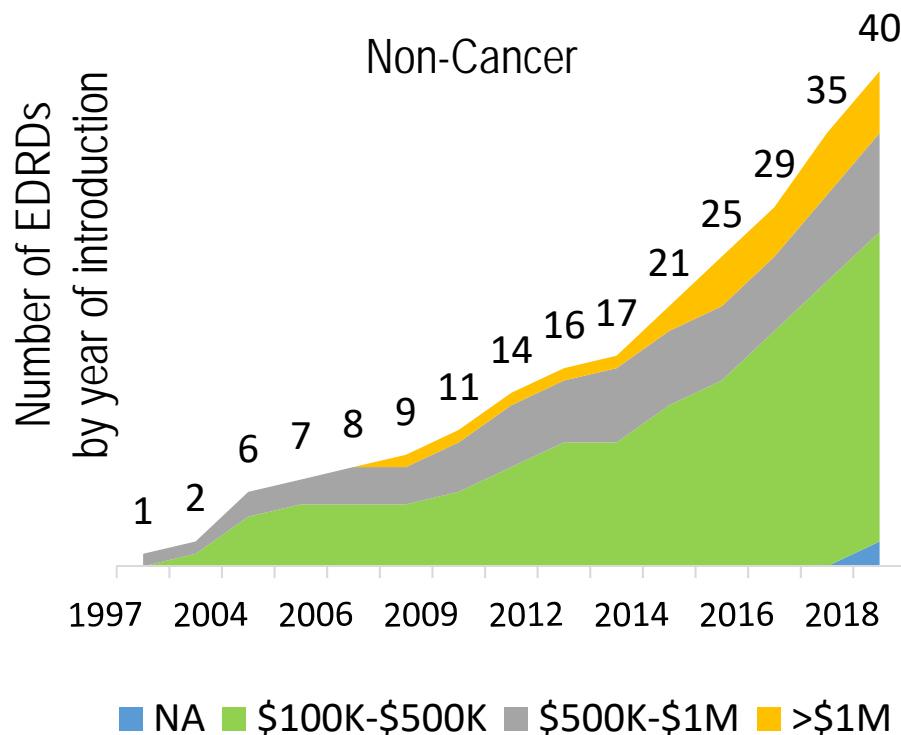
## CDER New Drugs Program: 2018 Update

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

# Increasing number of EDRDs being introduced

## 79 EDRDs approved in Canada by the end of 2018

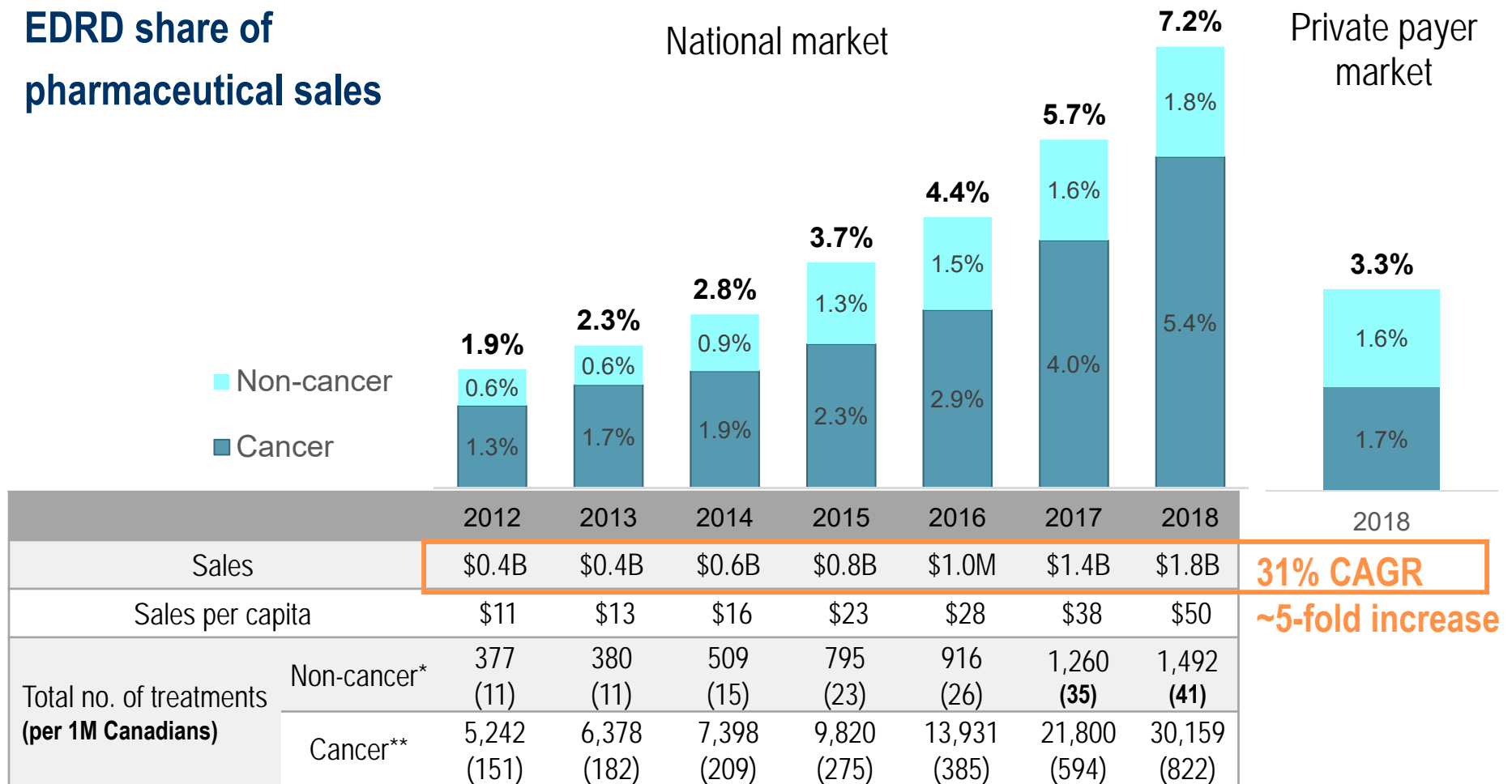
- Defined as having an orphan designation and an estimated treatment costs exceeding \$100,000 per year for non-cancer drugs, and \$7,500 per 28 days for cancer drugs



Source: PMPRB.

# EDRD – fastest growing market segment

## EDRD share of pharmaceutical sales

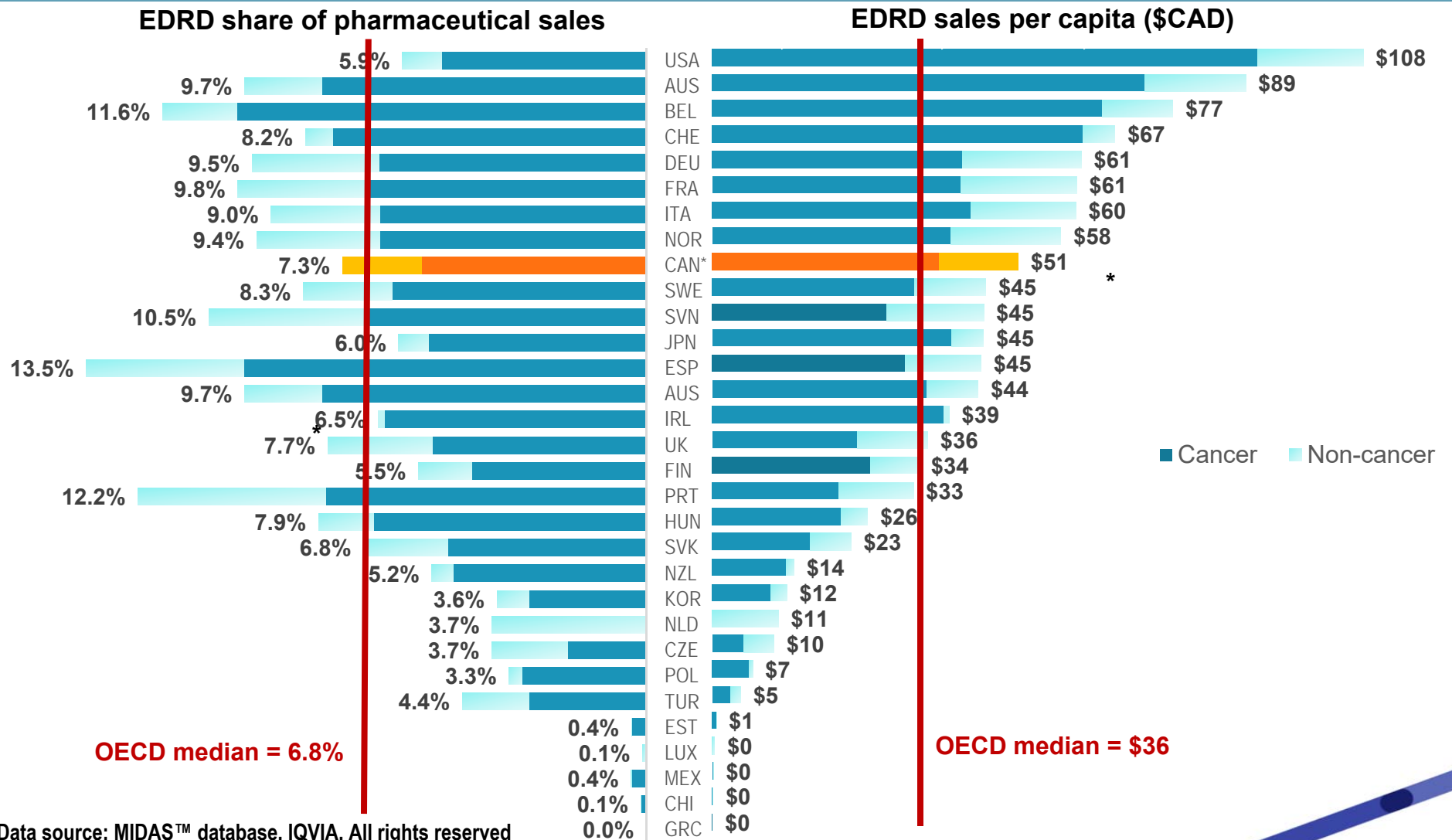


\*For non-cancer, the total number of treatments were estimated based on the annual treatment cost

\*\*For cancer, the total number of treatments were estimated based on the highest of (1) the average cost per beneficiary in private drug plans, or (2) the estimated cost of four courses of 28-day treatment.

Data source: PMPRB, MIDAS™ database, 2017, IQVIA. All rights reserved, IQVIA Private Pay Direct Drug Plan Database, 2018.

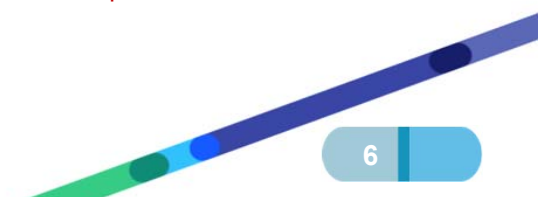
# EDRD spending in Canada above international norms



Data source: MIDAS™ database, IQVIA. All rights reserved




Note: Results based on list prices and not reflective of managed entry agreements

\* Estimated by complementing MIDAS™ results with PMPRB, and IQVIA Private Pay Direct Drug Plan Databases

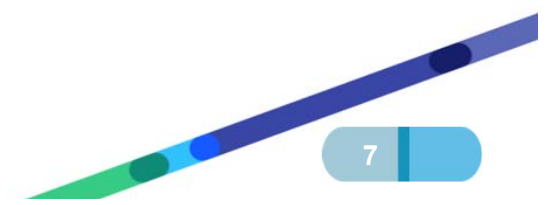


# Hypothesis: Rare disease drugs are expensive, because of the need to recuperate costs with fewer patients

Distribution of patented drugs by highest sales\* in the first 5 years

	\$10M-\$50M	\$50M-\$100M	\$100M+	$\Sigma$	Avg. sales per drug
<b>Lower-cost drugs</b> <\$10K annually 	25%	5%	5%	35%	\$21M
<b>Higher-cost drugs</b> ≥\$10K annually 	39%	11%	9%	59%	\$39M
<b>EDRDs**</b> 	34%	6%	10%	50%	\$29M

\* In 2018 dollars; drugs introduced in the last 20 years.  
 \*\* EDRDs include drugs with more than 2 years of sales.  
 Note: Results not reflective of managed entry agreements.



# Alternative hypothesis: Higher prices = higher sales

- (1) An EDRD is more likely to result in high sales than a lower-cost drug
- (2) A higher-cost drug is even more likely to result in high sales than a lower-cost drug

Price matters



*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*





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# THANK YOU

Patented Medicine Prices Review Board



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