Canadian Organization for Rare Disorders

Drawing the Blueprint for Canada's Rare Drug Program 2022 Winter 2021

Webinar 8

Laying a Factual Foundation

February 12, 2021

Countdown to Canada's Rare Drug Strategy 2022: Webinar Series

WEBINAR 7

Jan 29

@ 11AM (EST)

How Other Countries Provide Access to Rare Disease Drugs: What Canada can Learn … or Not

WEBINAR 8

Feb 12

@ 12PM (EST)

Laying a Factual Foundation for Pan-Canadian Rare Drug Program

WEBINAR 9

Feb 18

@ 6 PM (EST)

Everything You Need to Respond to Discussion Guide on National Strategy for High-Cost Drugs for Rare Diseases

WEBINAR 10

Feb 26

@ 12PM (EST)

Rare Disease Day: Celebrating Achievements that Support A Pan-Canadian Rare Drug Program

CORD Canadian Organization for Rare Disorders

Rare Disease Day Conference

From Draft to Action Plan

March 9 - 10, 2021

RARE DISEASE DAY®

Consultation Dates: January – September 2021

- Jan – Feb 2021: CORD ”Drawing the Blueprint” Webinar Series
- Jan – Mar 2021: Health Canada Public Consultations
- March 9 – 10, 2021: Rare Disease Day Conference ”From Draft to Action Plan”
- April 2021: Consolidated Design
- Jun – Jul 2021 Public Consultations
- Aug 2021: Collaborative Document

Jan 1, 2022: Ready to Launch 

Canadian Organization for Rare Disorders

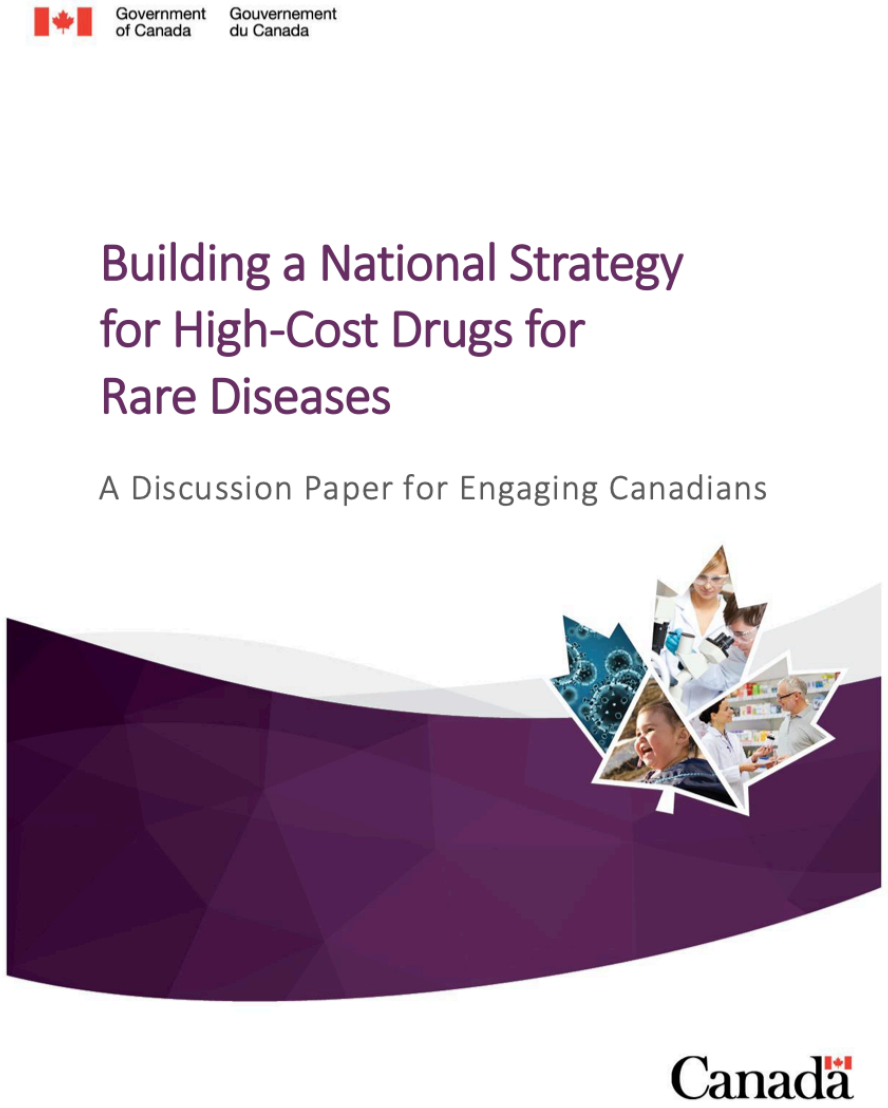
National Strategy for High-Cost Drugs for Rare Diseases Online Engagement

Government of Canada / Gouvernement du Canada

1. Complete the online **questionnaire**
2. Send a written submission via **email** or **mail**
3. Participate in a virtual **public town hall**

Public Town Hall #1	February 9, 2021	3:00 – 4:30pm	Bilingual
Public Town Hall #2	February 25, 2021*	7:00 – 8:30pm	Bilingual
Public Town Hall #3	March 3, 2021*	1:00 – 2:30pm	French
Public Town Hall #4	March 12, 2021	10:00 – 11:30am	Bilingual
Public Town Hall #5	March 23, 2021	3:00 – 4:30pm	Bilingual

To find out more about this consultation on the National Strategy for High-Cost Drugs for Rare Diseases, please click on the following link: https://canada.ca/en/health-canada/programs/consultation-national-strategy-high-cost-drugs-rare-diseases-online-engagement.html



Webinar 8: Laying a Factual Foundation for a Pan-Canadian Drug Program

Separating Fact from Fiction: Status of Access to Rare Drugs in Canada

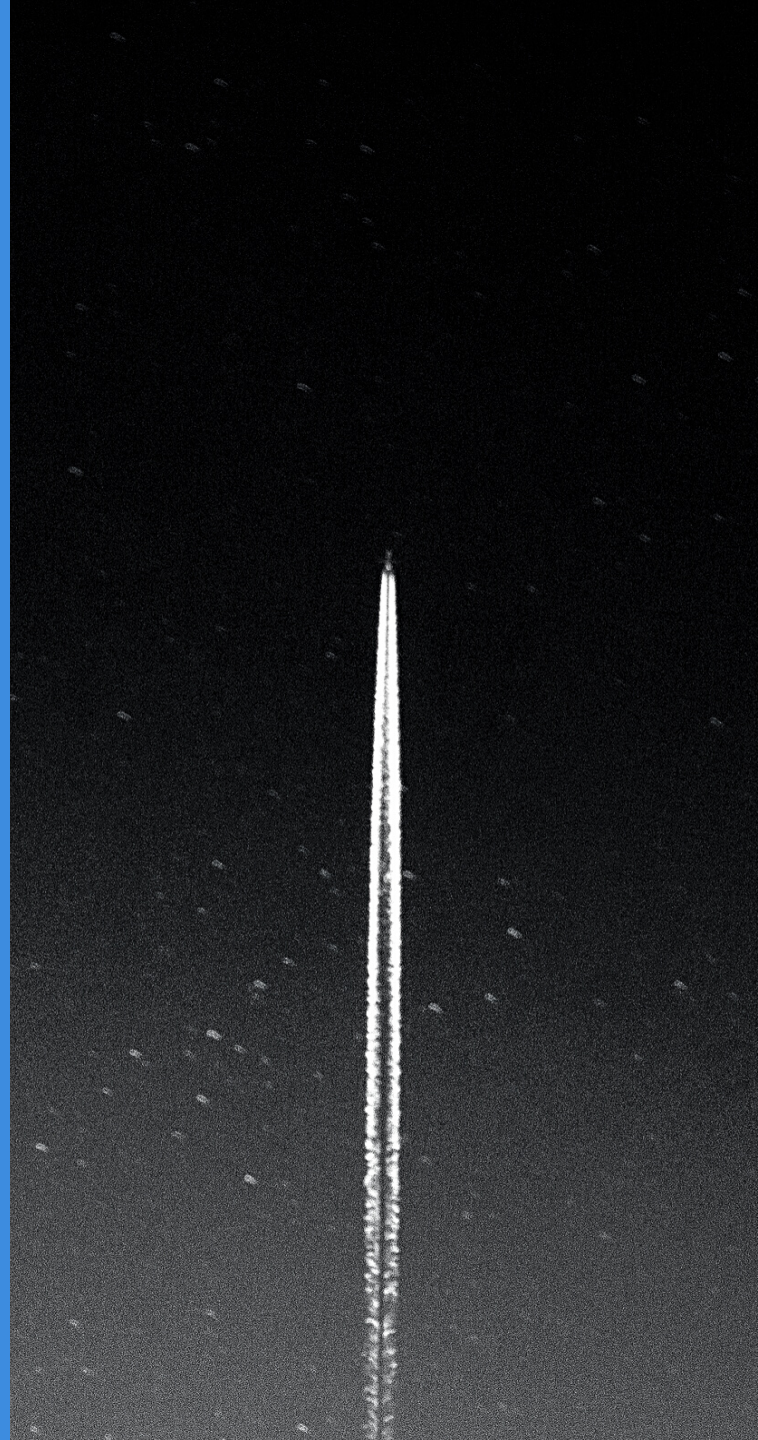
Webinar 8: Lead panel

Agenda

Canada’s Long Journey To Rare Drug Strategy
Separating Fact from Fiction

- Interactive Polls
- Expert Commentators
- All Participants

Moderator: Durhane Wong-Rieger, CORD



Canadian Organization
for Rare Disorders

CORD Rare Drug Program
Webinar 8 Polls

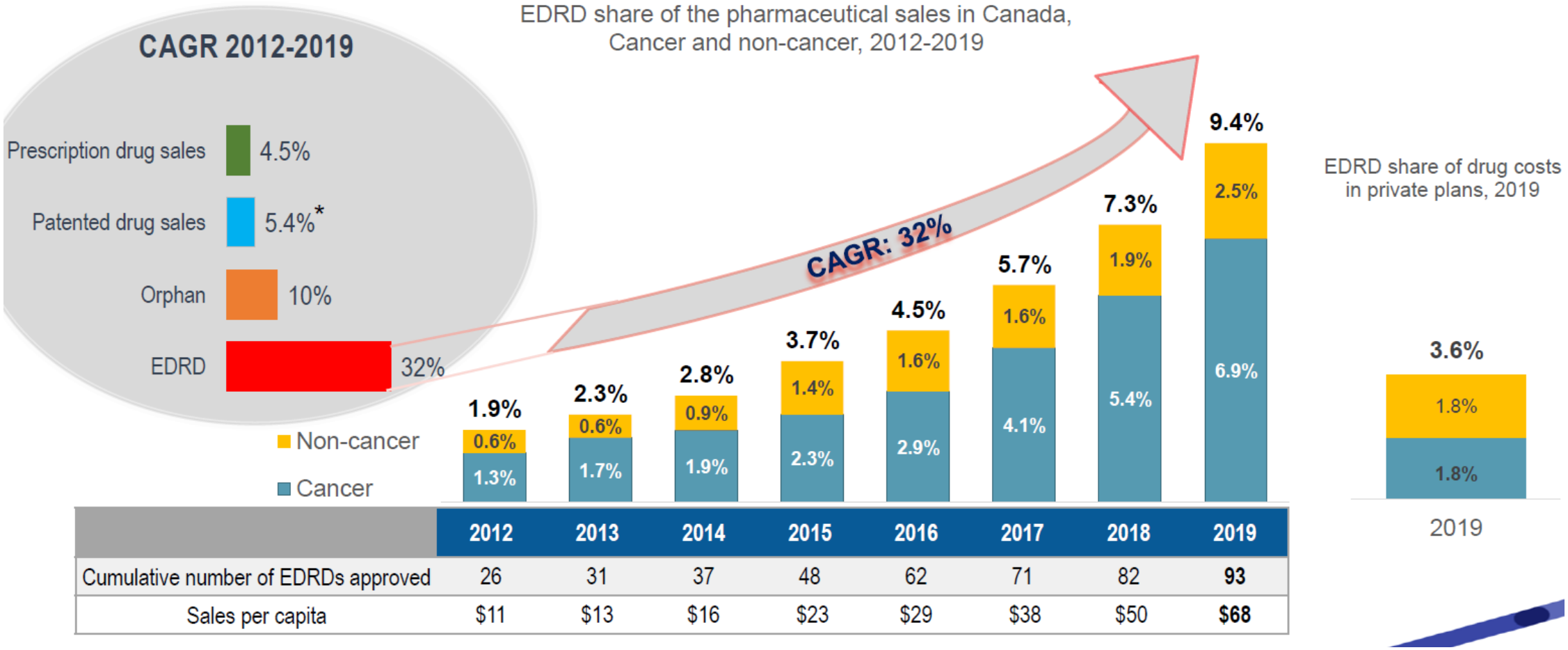
Canadian Organization for Rare Disorders

Fact or Fiction 1:

1. CLAIM: EDRDs is fastest growing market sector and “pushing the limits of affordability.” Which of following is FACT?

- >70% of spending identified as “EDRD” is for oncology drugs for cancers (rare & nonrare)
- 2.5% of public drug spend is for (non-oncology) EDRDs
- <2% of private drug spend is for EDRDs, not including oncology
- Total Canadian drug budget for expensive and non-expensive DRDs was ~2%
- None of the above
- All of the above

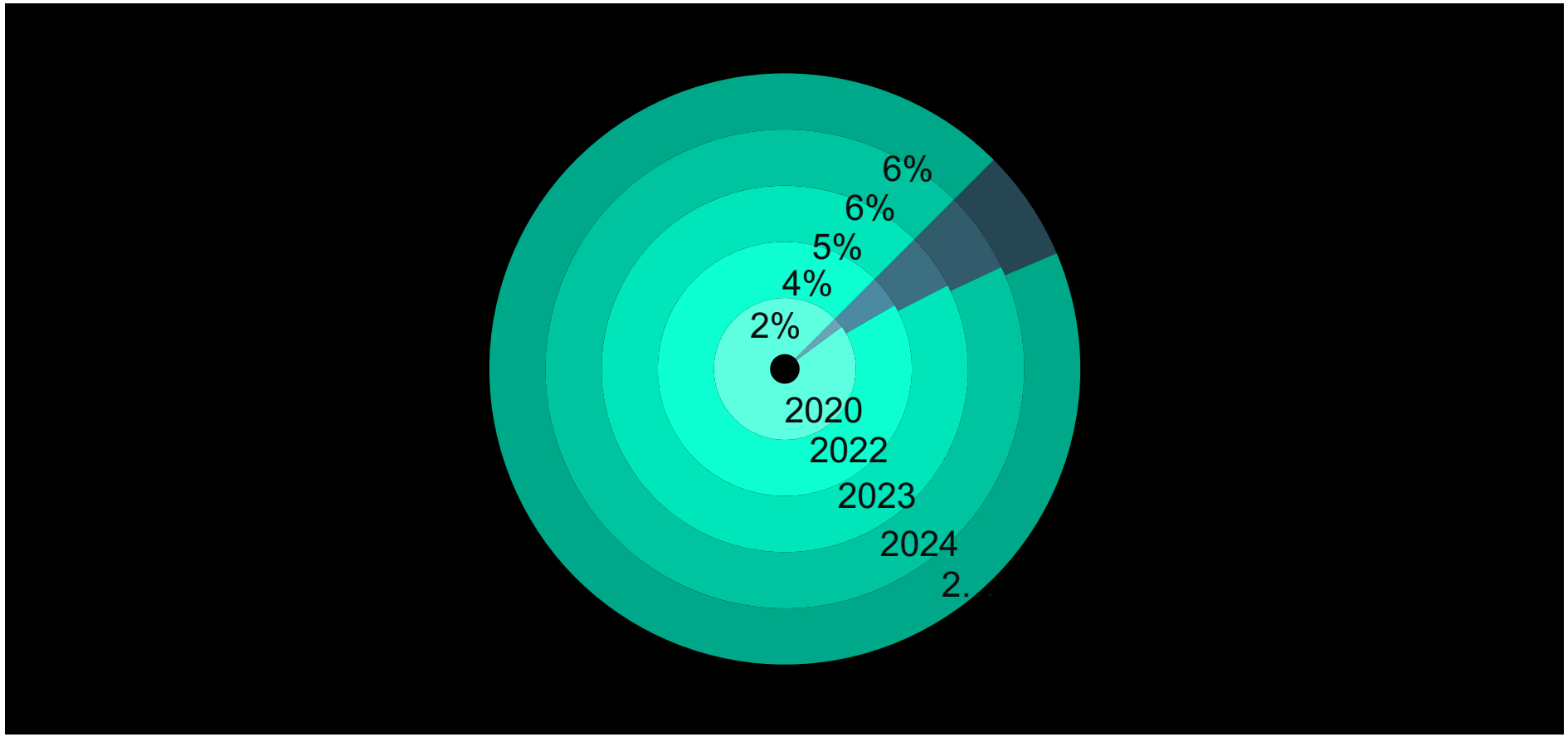
“EDRDs are the fastest growing market segment”



	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**

From: PMPRB Research Webinar. Insight into the spending on expensive drugs for rare diseases. June 23, 2020. Page 10.

The total expenditure on DRDs was ~2% of the total drug budget in 2019



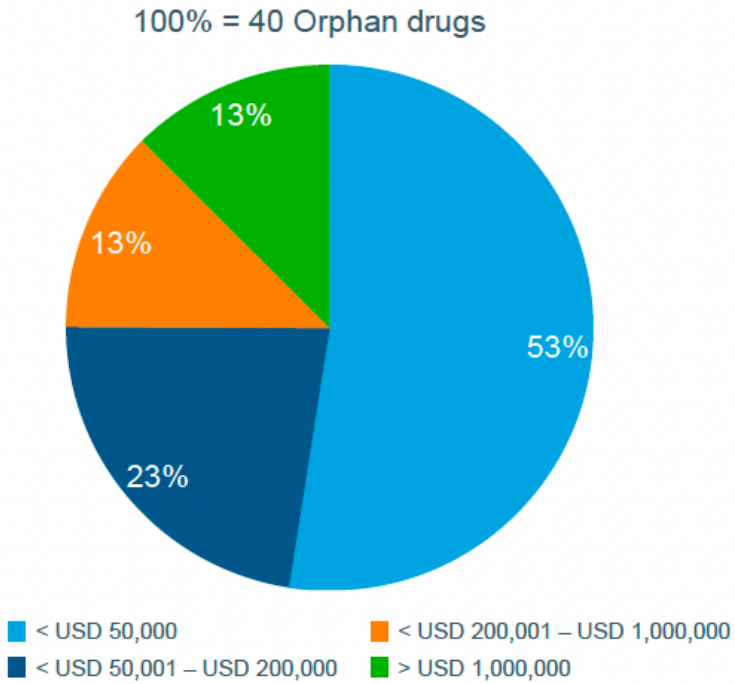
Fact or Fiction 2:

2. Which of the following statements about budget impact is NOT TRUE?

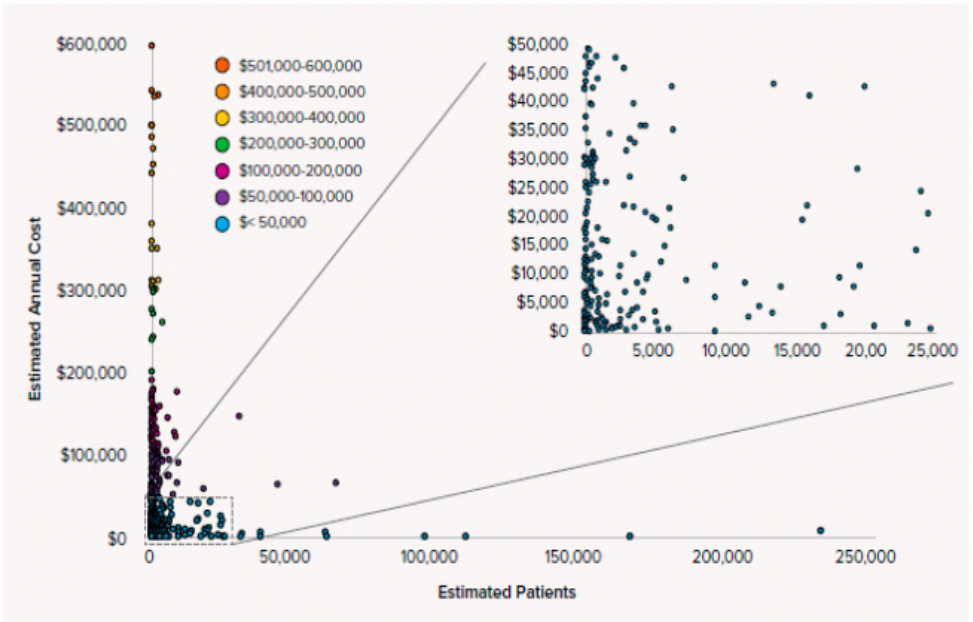
- Annual budget impact of most orphan drugs < $50k in Europe and <$35k in USA.
- Annual per patient cost of most RD drugs is $100,000 to $2 million in Canada
- Oncology precision meds & orphan drug sales growing at same rate (11.8% over 5 years)
- The smaller # of patients treated by RD drug, higher per patient $.

The annual cost of the majority of orphan medicines in Europe is below $50,000, while the median annual cost per OD in the US is ~$32,000

Orphan Drug Distribution by Annual Cost (average list price by indication), EU 2017

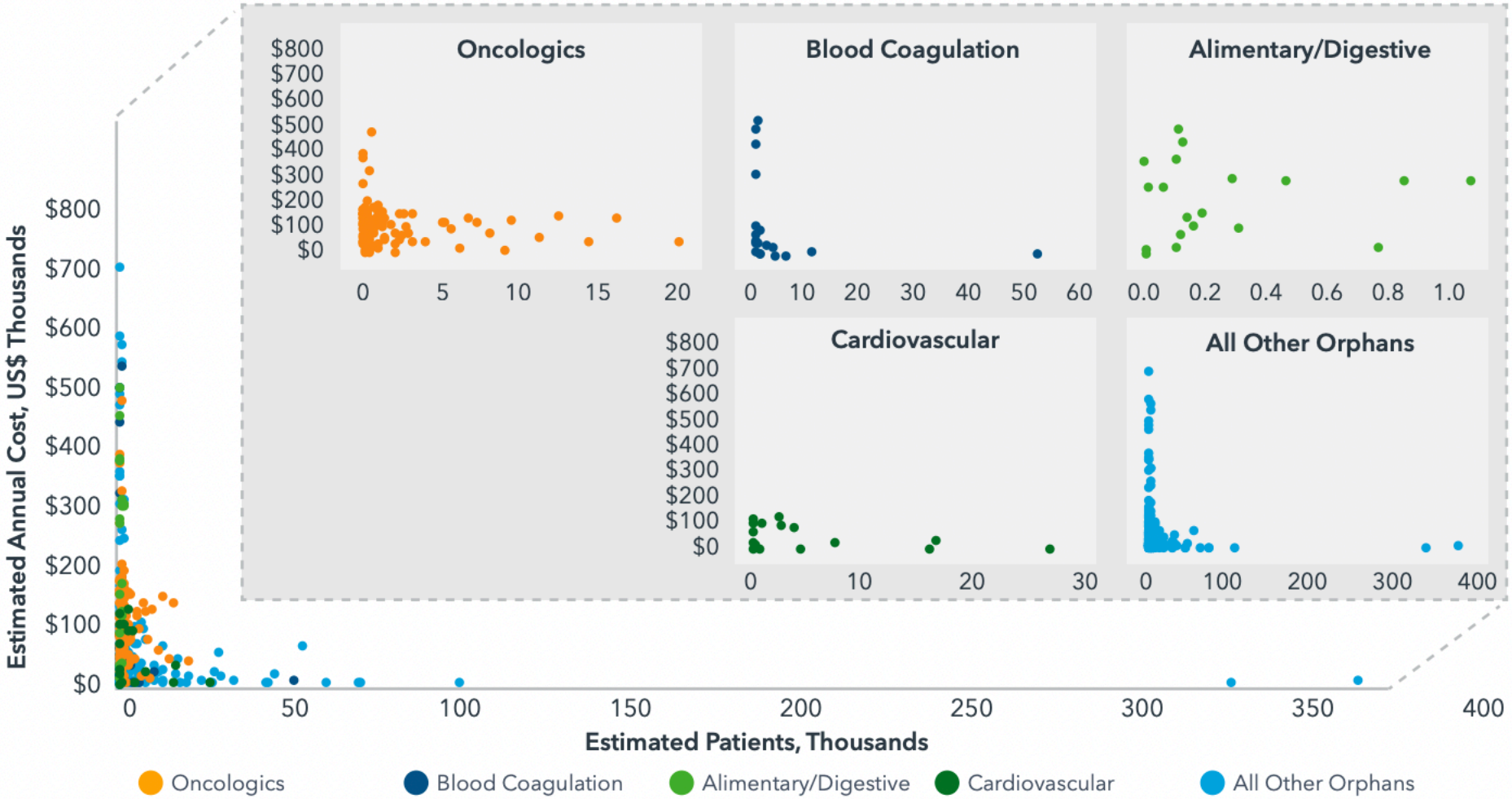


Orphan Drug Distribution by Annual Cost, US, 2016



Price sources:
1. IQVIA Pricing Insights
2. IQVIA analysis

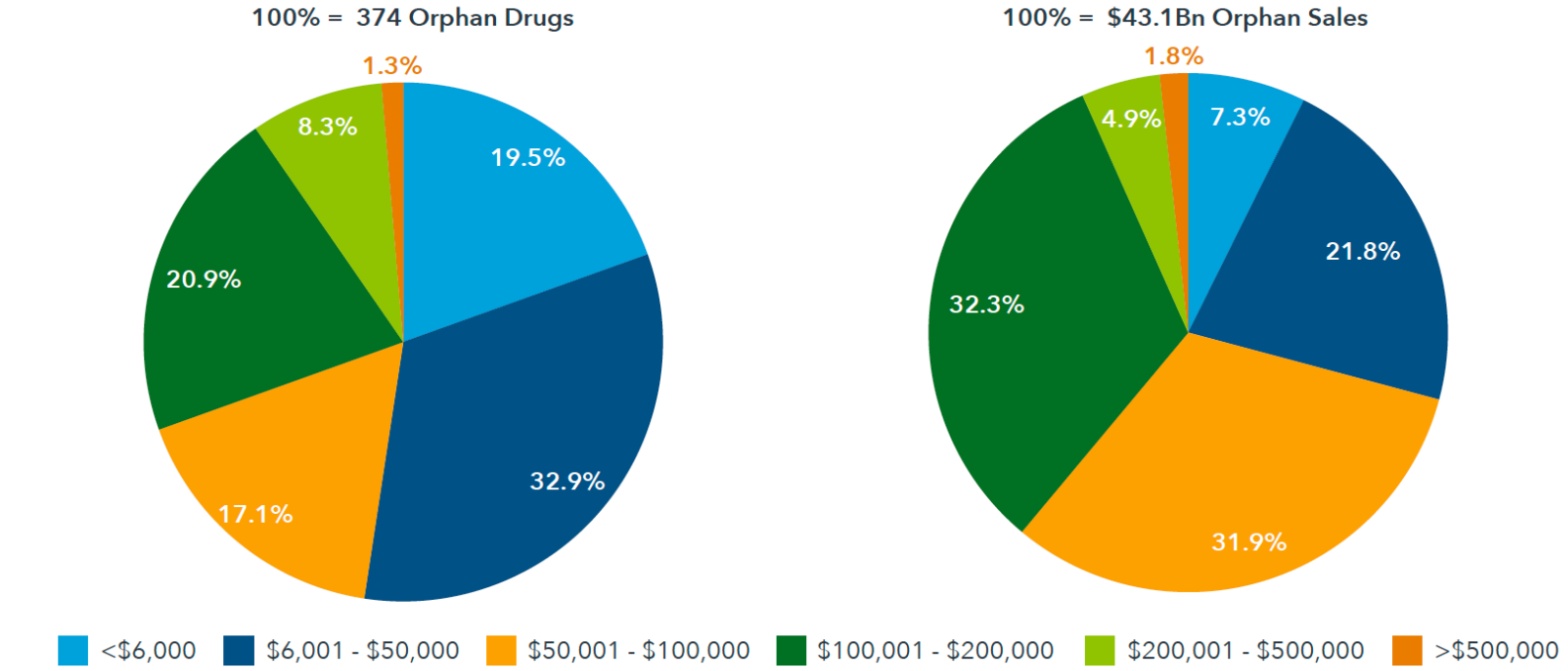
Exhibit 7: Estimated Target Patient Population Versus Cost for Orphan Drugs in the United States in 2017, US$ Thousands



Source: IQVIA National Sales Perspectives, Jan 2018; FDA Orphan Drugs Database, accessed Sep 2018; IQVIA Institute, Sep 2018

Note: Though scales vary, all x-axes of charts within the zoom box display the number of patients in thousands.

Exhibit 21: Orphan Drug Counts and Sales by Annual per Patient Cost in the United States in 2017



Source: IQVIA National Sales Perspectives, Jan 2018; FDA Orphan Drugs Database, accessed Sep 2018; IQVIA Institute, Sep 2018

The annual cost per patient for orphan drugs also varies widely. About 20% of the drugs (n=73) are priced at less than $6,000 per year, and they contribute 7.3% of total spending on orphan drugs. About 1.3% of orphan drugs are priced in excess of $500,000 per year (n=5), but they only account for 1.8% of orphan drug spending due to relatively few patients being treated with these medicines (see Exhibit 21). Examples include Actimmune, Brineura and Soliris.

Source: *Precedence Research*

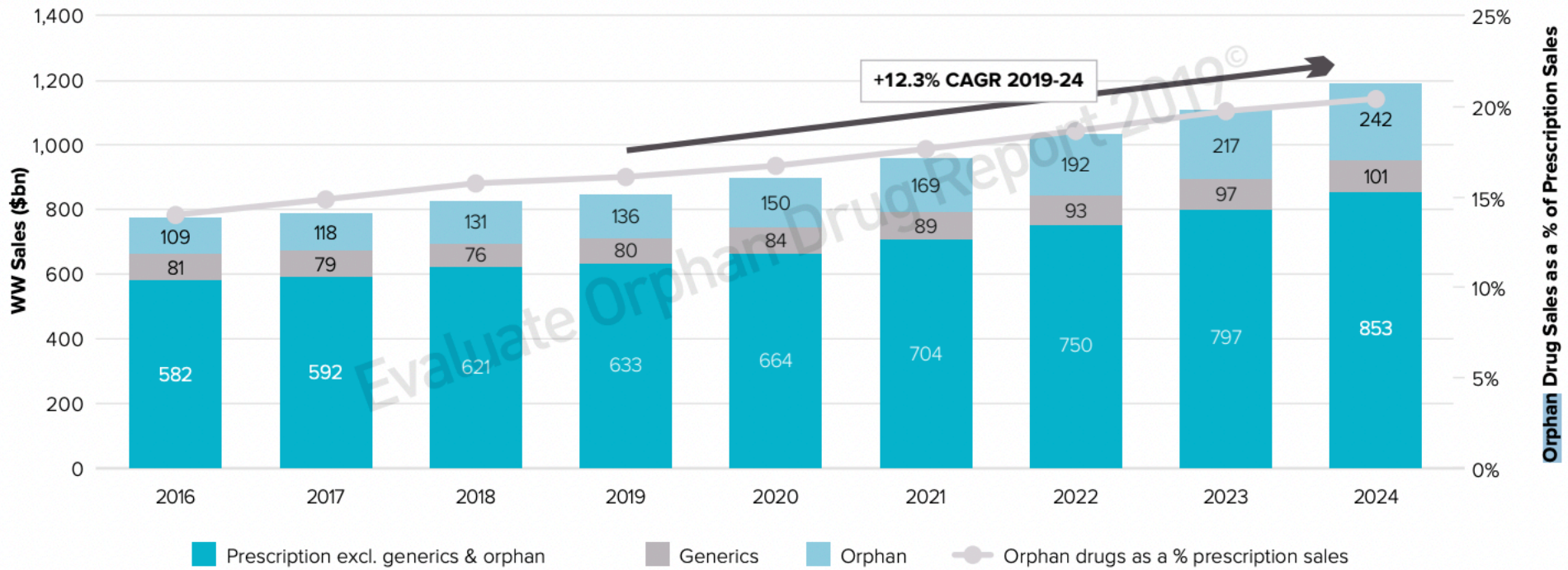
November 30, 2020 09:43 ET

Precision Medicine Market Poised to Grow at 11.5% By 20227

According to Precedence Research, the global precision medicine market is poised to grow at a CAGR of 11.5% during the forecast period 2020 to 2027.

OTTAWA, Nov. 30, 2020 (GLOBE NEWSWIRE) -- The global **precision medicine market** value surpassed USD 59.16 billion in 2019 and expected to reach USD 141.33 billion by 2027.

Figure 2: Worldwide Orphan Drug Sales & Share of Prescription Drug Market (2016-2024)



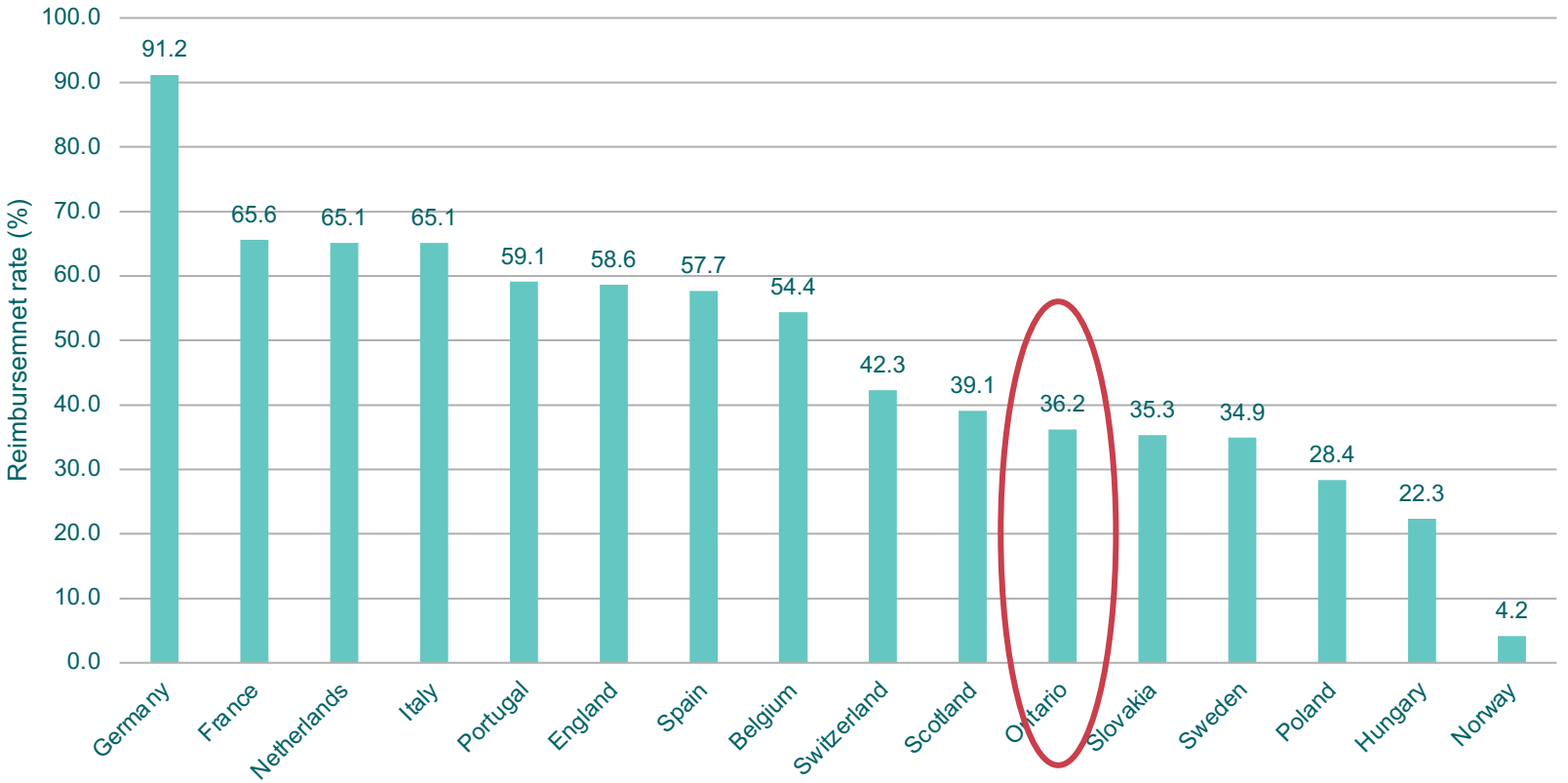
Fact or Fiction 3:

Which of the following statements is TRUE about availability of rare disease drugs in Canada?

- 80% of orphan drugs approved in the EU or USA are available in Canada within two years
- Half of rare disease drugs funded in top 6 EU countries are reimbursed in Canada
- Half of new drug approvals in USA in 2019-20 were approved in Canada by 2020.
- Most Canadians with private drug plans can access most approved RD drugs.

Rate of reimbursement of OMPs (2001 – 2019) %

- The country with the highest level of coverage is Germany (with over 90%), followed by France, the Netherlands and Italy (with around 65%)
- The three countries with the lowest level of coverage are Poland, Hungary and Norway (below 30%)
- **Canada (represented by Ontario) had a 36% reimbursement rate.**



	Ontario	Alberta	Quebec	British Columbia
Number of OMPs Health Canada authorised	125	124	124	124
#OMPs with a decision on use	92	85	96	79
% of OMPs EMA authorised	43%	40%	45%	37%
% of OMPs Health Canada authorised	74%	69%	77%	64%
#OMPs Reimbursed	78	55	61	49
% of OMPs EMA authorised	36%	26%	28%	23%
% of OMPs Health Canada authorised	62%	44%	49%	40%
#OMPs fully reimbursed	74	53	59	47
#OMPs partially reimbursed	4	2	2	2

TABLE 2 - OMP REIMBURSEMENT IN CANADA

Drug Name	FDA Approval Date	Submitted to HC?	ubmission/Approval I
Qinlock	15-May-20	yes	*NDS Mar 20, App Ju*
Retevmo	08-May-20	no	
Tabrecta	06-May-20	no	
Ongentys	24-Apr-20	no	
Trodelvy	22-Apr-20	no	
Pemazyre	17-Apr-20	no	
Tukysa	*17-Apr-20*	*yes*	*NDS Feb 20 App Jun 2*
Koselugo	10-Apr-20	no	
Zeposia	25-Mar-20	yes	NDS Dec 19
Isturisa	06-Mar-20	no	
Sarclisa	03-Mar-20	yes	NDS Jul 19 App Apr
Nurtec ODT	27-Feb-20	no	
Barhemsys	26-Feb-20	no	
Vyepti	21-Feb-20	no	
Nexletol	21-Feb-20	no	
Pizensy	12-Feb-20	no	
Tazverik	23-Jan-20	no	
Tepezza	21-Jan-20	no	
Ayvakit	09-Jan-20	no	
Accrufer	25-Jul-19	no	
Adakveo	15-Nov-19	no	
Aklief	04-Oct-19	yes	NDS Jan 19 App Nov 2
Balversa	12-Apr-19	yes	NDS Mar 19 App Oct 1
Beovu	07-Oct-19	yes	NDS May 19; App Mar
Brukinsa	14-Nov-19	no	
Cablivi	06-Feb-19	yes	NDS Aug 19 App Feb 2
Calquence	21-Nov-19	yes	NDS Mar 18 App Nov 1
Caplyta	20-Dec-19	no	
Dayvigo	20-Dec-19	yes	NDS Jan 20
Egaten	13-Feb-19	no	
Enhertu	20-Dec-19	yes	NDS (17/18; SNDS Sep
Evenity	09-Apr-19	yes	NDS Oct 16 App Jun 1
ExEm Foam	07-Nov-19	no	
Fetroja	14-Nov-19	no	
Ga-68-DOTATOC	21-Aug-19	no	
Givlaari	20-Nov-19	no	

Ibsrela	12-Sep-19	yes	NDS Jun 19 App Apr 2
Inrebic	16-Aug-19	yes	NDS Oct 19 App Sep 2
Jeuveau	01-Feb-19	yes	NDS Oct 17, App Aug 1
Keytruda-Lenvima Combo	18-Sep-19	yes	App Sep 19
Mayzent	26-Mar-19	yes	NDS Feb 19 App Feb 2
Nourianz	27-Aug-19	no	
Nubeqa	30-Jul-19	yes	NDS Ap 2019 App Feb
Oxbryta	25-Nov-19	no	
Padcev	18-Dec-19	no	
Piqray	24-May-19	yes	NSS May 19; App Mar
Polivy	10-Jun-19	yes	NDS Nov 19 App Jul 20
Reblozyl	08-Nov-19	no	
Recarbrio	16-Jul-19	no	
Reyvow	11-Oct-19	no	
Rinvoq	16-Aug-19	yes	NDS Mar 19 App Dec 1
Rozlytrek	15-Aug-19	yes	NDS Jun 19 App Feb 2
Scenesse	08-Oct-19	no	
Skyrizi	23-Apr-19	yes	NDS May 18, App April
Sunosi	20-Mar-19	no	
TissueBlue	20-Dec-19	no	
Trikafta	21-Oct-19	no	
Turalio	02-Aug-19	no	
Ubrelvy	23-Dec-19	No	
Vyleesi	21-Jun-19	no	
Vyndaqel	03-May-19	yes	NDS Jul 19 App Jan 20
Vyondys 53	12-Dec-19	no	
Wakix	14-Aug-19	no	
Xcopri	21-Nov-19	no	
Xenleta	19-Aug-19	yes	NDS Jan 20
Xpovio	03-Jul-19	no	
Zulresso	19-Mar-19	no	
	14-Aug-19	no	
Aemcolo	16-Nov-18	no	
Ajovy	14-Sep-18	yes	NDS Jun 19 App Aug 2
Asparlas	20-Dec-18	no	
	HC Submission	26	21
	No HC Submission	45	

Fact or Fiction 4:

Which of these rare drug pricing solutions supports innovation, access, and sustainable budgets?

- Increase ICER from $50k/QALY for common drugs to $200k/QALY for innovative drugs
- Set maximum profit for rare disease drugs; if exceed, require rebate.
- Offer incentives for R&D, reduce fees & time to approval; support risk-sharing managed access
- Start access through private plans; negotiate province by province for public access

Fact or Fiction 5:

Which of the following is TRUE about the “time to reimbursement” for rare disease drug in Canada?

- For 2001-19, avg time to reimburse in Ontario < 2years
- Province w/most reimbursed RD drugs has shortest time to reimbursement.
- Since pCPA, time from CADTH recommendation to reimbursement is same across provinces
- Compared to EU, Canada ranks above only Poland, Slovakia, Hungary, and Portugal in timeliness to access

Average time to reimbursement (2001 – 2019)

'Time to reimbursement' is defined as the average time in days from marketing authorisation to available reimbursement decisions date.

Germany has shortest timelines to reimbursement, followed by Switzerland and Scotland (less than 500 days), Italy, Spain and Sweden (less than 600 days)

- **Canada (represented by Ontario) had an average time to reimburse of just under 800 days**
- Poland, Slovakia and Hungary have low rates of reimbursement and are also associated with the longest delays (1200 days and higher)

N.B. Time to reimbursement for Ontario is calculated using the Health Canada marketing authorisation date, not EMA date.
Time to reimbursement for Switzerland is calculated using Swissmedic marketing authorisation date.