

From Accelerated Access to Lifetime Impact

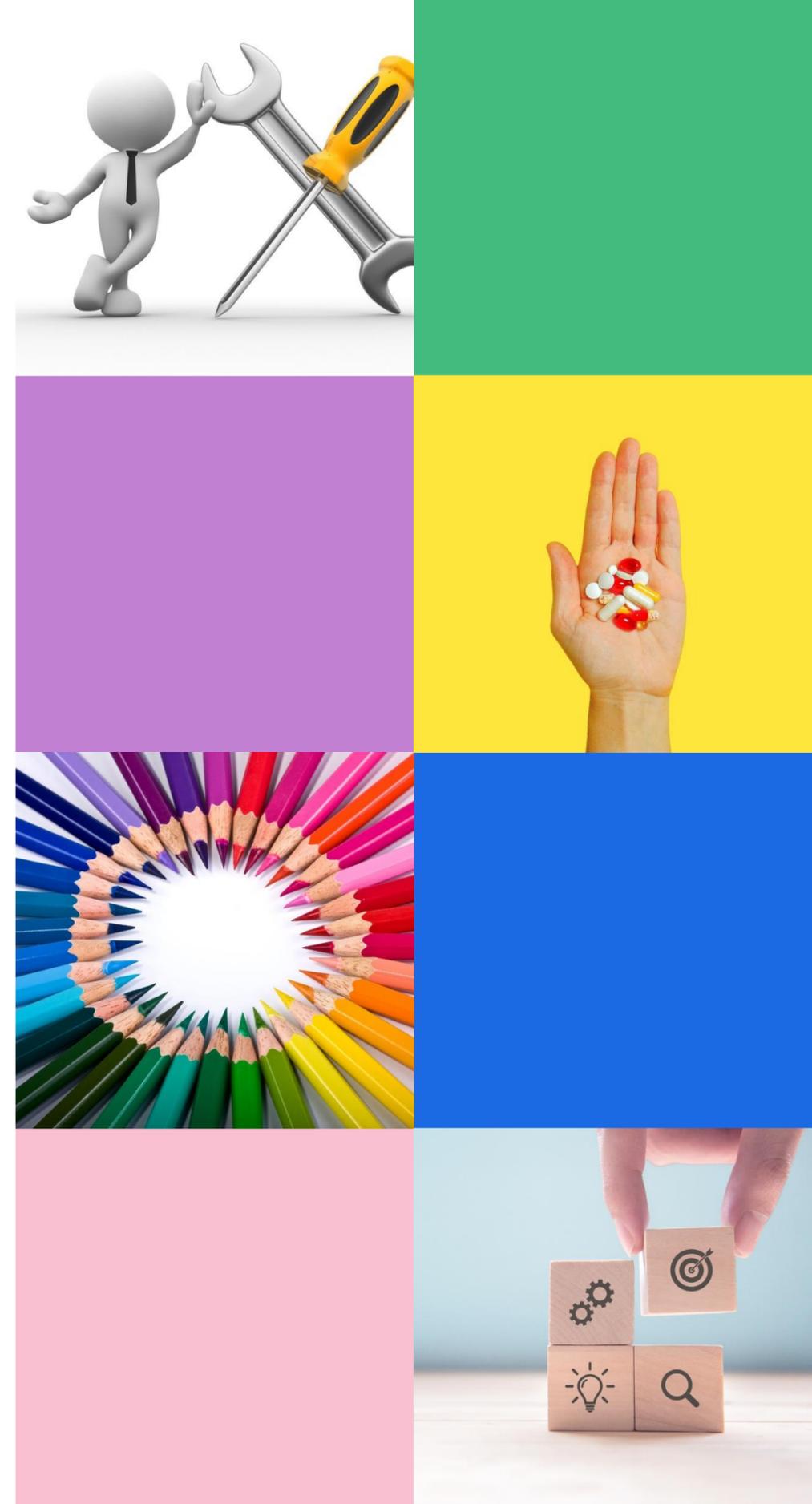
Canada's Rare Drug Strategy at 18 Months

February 2026

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National Drugs for Rare Diseases Strategy overview



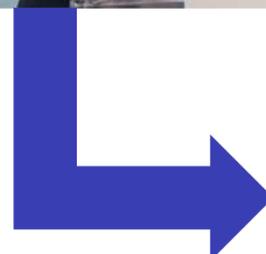
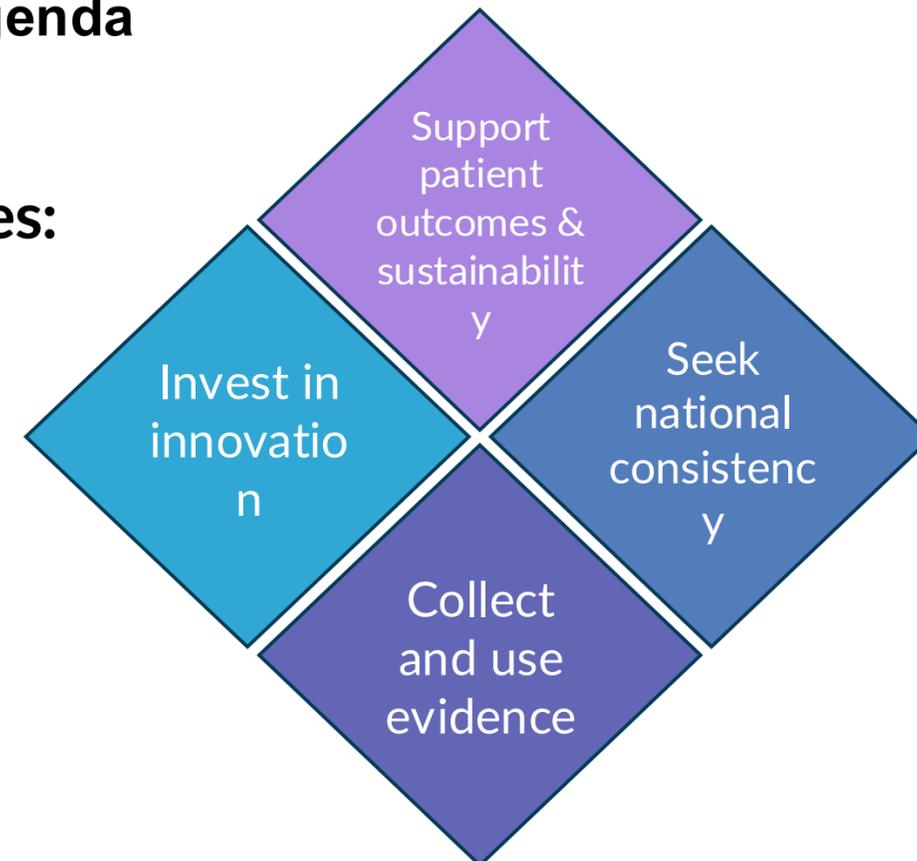
Government of Canada improves access to affordable and effective drugs for rare diseases



National Strategy for Drugs for Rare Diseases

- The goal of the first three-year phase is to increase access to, and affordability of, effective drugs for rare diseases, which will contribute to improving the health of patients across Canada
- **Lessons learned will be incorporated into future phases, staying aligned with the government's broader pharmaceutical agenda**

Objectives:

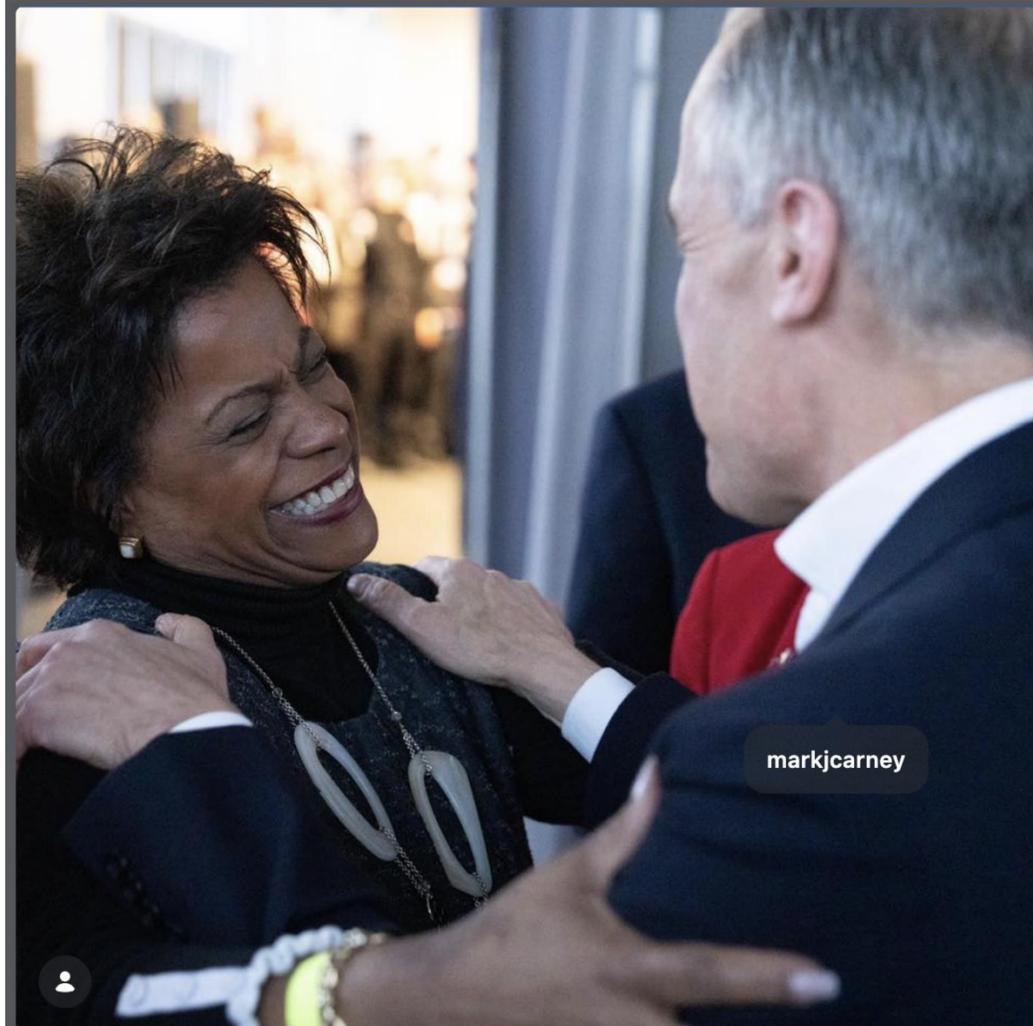


new three-year, \$1.5-billion national rare disease treatment strategy announced in March 2023

18 Months Into Canada's Rare Disease Drug Strategy



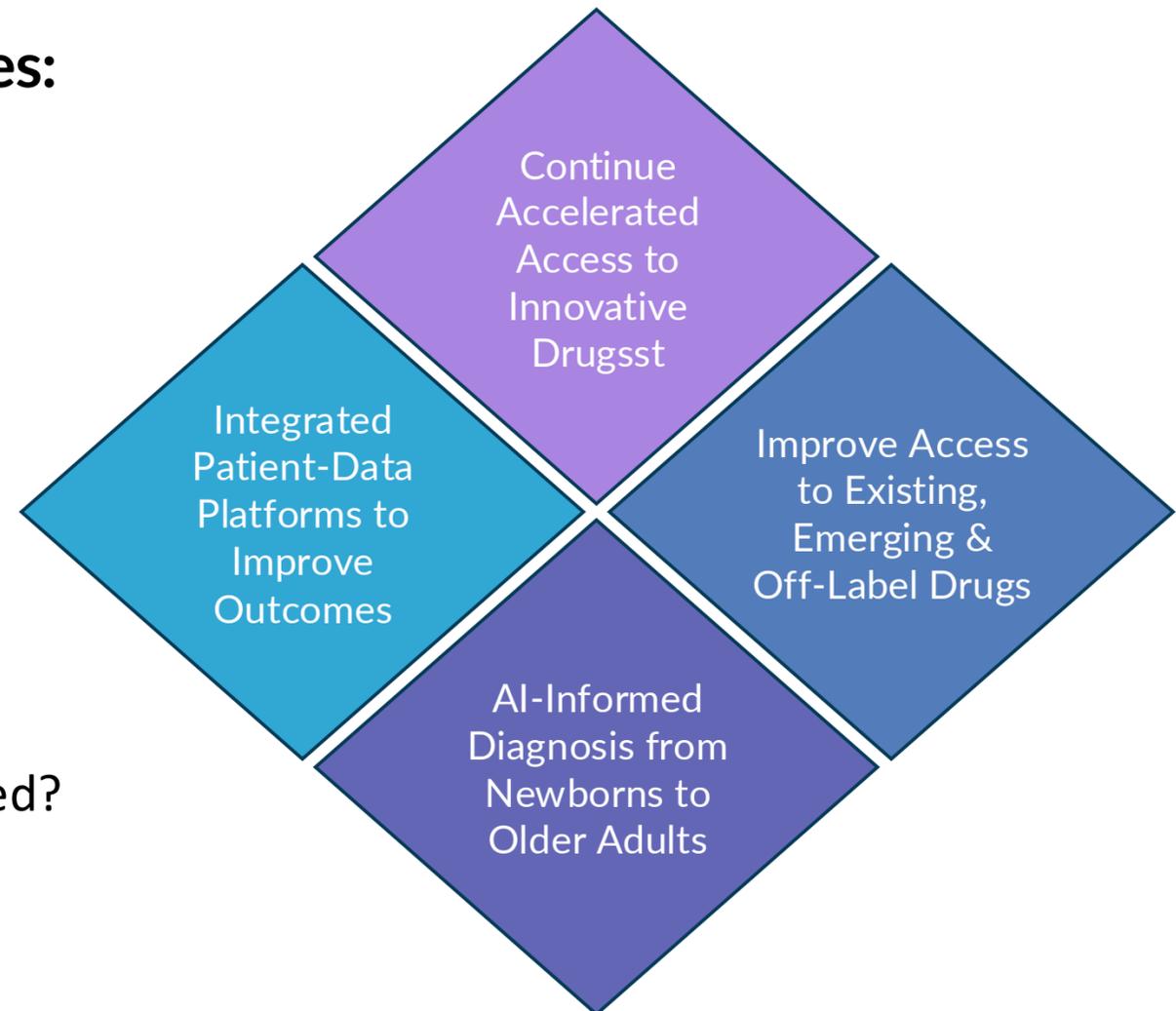
Government of Canada improves access to affordable and effective drugs for rare diseases



Real Acceleration. Challenge to Deliver Real Outcomes

- First 1.5 years: The Rare Disease Drug Strategy (RDDS) is demonstrating measurable progress in drug access.
- 2nd 1.5 years: The next phase must demonstrate value of access and measurable impact on patient outcomes
- 2027 and beyond: This prepare for Phase 2: investment in entire patient journey (diagnosis, care, and management) to achieve equitable outcomes.

Objectives:



\$1.5-billion expires April 2027: What have we achieved?
What else must we do—to optimize value?

RDDS Common List

Drug Brand	Manufacturer	Indications	CDA Interval	CDA to pCPA	pCPA Interval	HC-LOI	CDA - LOI	CDA ICER	Discount
Yescarta	Gilead	diffuse/high grade large B-cell lymphoma	259	215	346	2026	820	\$404,418	45%
Poteligeo	Kyowa Kirin	mycosis fungoides and Sézary syndrome	333	129	450	813	912	UK	51%
Oxlumo	Alnylam	primary hyperoxaluria type 1 (PH1)	274	212	231	781	717	\$2,171,687	95%
Welireg	Merck	von Hippel-Landau	221	97	267	781	585	\$360,193	83%
Koselugo	Alexion	neurofibromatosis type 1 (NF1)	436	307	252	831	995	\$426,286	88%
Koselugo	Alexion	neurofibromatosis type 1 (NF1)	436	307	252	831	995	\$294,751	83%
Eplinky	AbbVie	diffuse/high grade large B-cell lymphoma	200	-49	98	341	249	\$87,735	45%
Sohonos	Ipsen	fibrodysplasia ossificans progressive (FOP)	515	220	402	1137	622	\$13,055,900	99%
Imcivree	Rhythm Pharmaceuticals	Bardet-Biedl syndrome (BC)	150	310	295	755	207	\$2,336,431	98%
AVERAGE			313	192	286	921	685	\$21,957,31	76%

RDDS Common List: Measurable System Acceleration



Average timelines (Common List data):

- 313 days – CDA interval
- 192 days – CDA → pCPA
- 286 days – pCPA interval
- 685 days – CDA → LOI
- 921 days – HC-LOI

Average negotiated discount: 76%



Significant price reductions + faster negotiation = meaningful fiscal stewardship.



The machinery is moving — this deserves recognition.

Provincial Update: Alignment is Happening: (February 2026)

Province/Territory	Date Signed	3-Year Budget	Coverage
 British Columbia	July 23, 2024	\$194M	<i>Poteligeo, Oxlumo, Epkinly, Welireg, Yescarta, Koselugo, Sohonos, Imcivree, Evkeeza</i>
 Newfoundland & Labrador	Nov 15, 2024	\$22M	<i>Poteligeo, Oxlumo, Epkinly, Welireg, Yescarta, Koselugo, Sohonos, Imcivree</i>
 Alberta	Dec 5, 2024	\$162M	<i>Poteligeo, Oxlumo, Epkinly, Welireg, Yescarta, Koselugo, Sohonos, Evkeeza</i>
 Saskatchewan	Jan 10, 2025	\$40M	<i>Poteligeo, Oxlumo, Epkinly, Welireg, Yescarta, Koselugo, Sohonos, Imcivree, Evkeeza</i>
 New Brunswick	Jan 15, 2025	\$32M	<i>Poteligeo, Oxlumo, Epkinly, Welireg, Koselugo</i>
 Ontario	Jan 24, 2025	\$535M	<i>Poteligeo, Oxlumo, Epkinly, Welireg, Yescarta, Koselugo, Sohonos, Imcivree, Evkeeza</i>
 Manitoba	Feb 27, 2025	\$48M	<i>Poteligeo, Oxlumo, Epkinly, Welireg, Yescarta</i>
 Prince Edward Island	Mar 7, 2025	\$3.43M	<i>Poteligeo, Epkinly, Koselugo</i>
 Northwest Territories	Mar 13, 2025	\$7.8M	<i>Poteligeo</i>
 Nunavut	Mar 13, 2025	\$8.5M	<i>Poteligeo, Epkinly, Welireg, Yescarta</i>
 Yukon	Mar 13, 2025	\$8.5M	<i>Yescarta</i>
 Nova Scotia	Mar 20, 2025	\$39M	<i>Poteligeo, Oxlumo, Epkinly, Welireg, Yescarta, Koselugo, Sohonos, Imcivree, Evkeeza</i>
 Quebec	Mar 21, 2025	\$305M	Program focus

- 9 provinces + 3 territories listing ≥1 Common List drug
- 4 provinces listing 9 drugs
- Listings now occurring almost immediately after agreements

National coordination is improving!

But:
No public evaluation yet on how the **\$1.4B** is being allocated or spent provincially.



A JOURNEY THROUGH LIFE WITH A RARE DISEASE

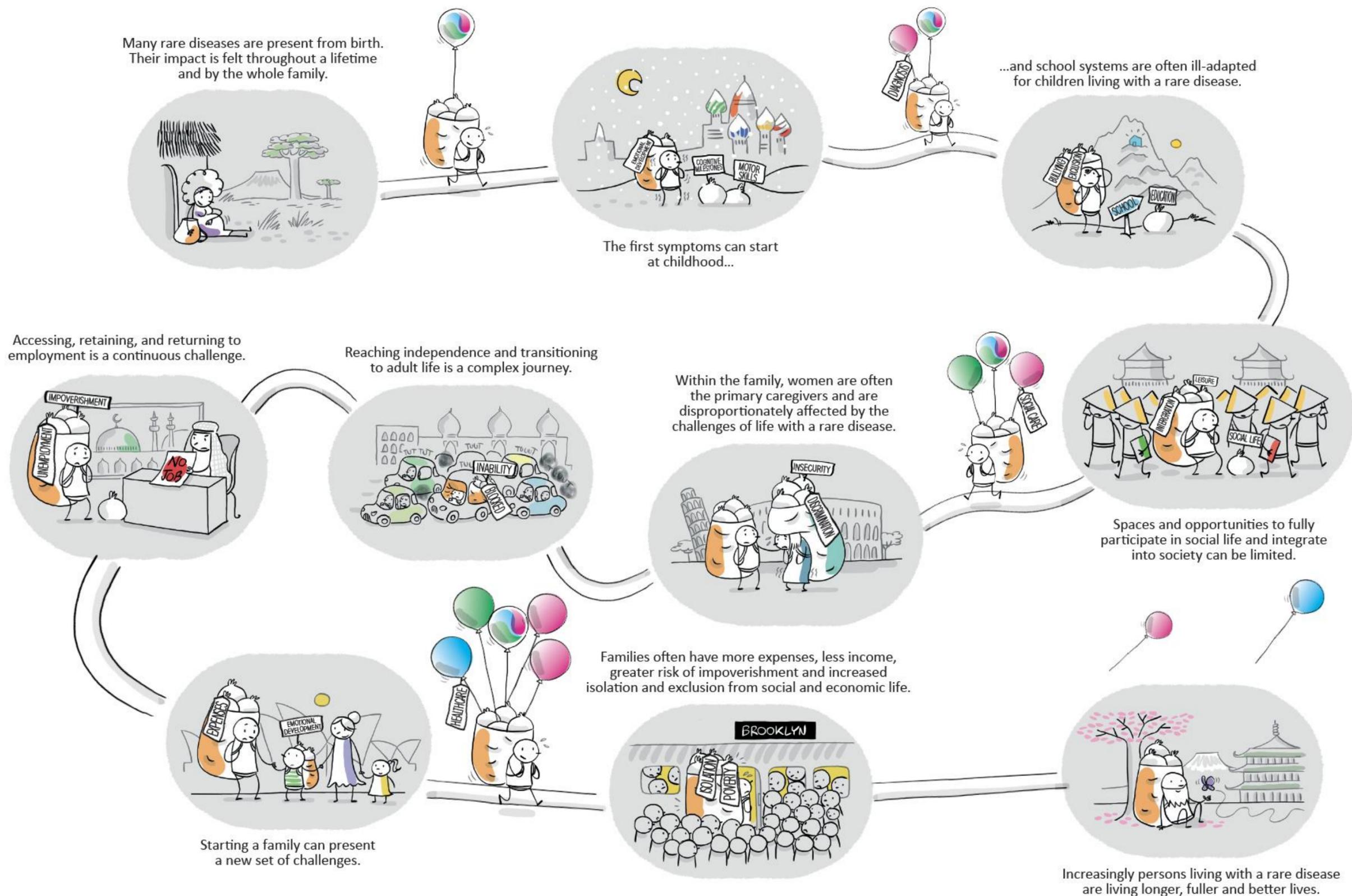
Acceleration Is Necessary — But Not Sufficient

Listing ≠ Access ≠ Outcomes

Effective rare disease care requires:

1. Diagnosis
2. Referral pathways
3. Specialist expertise
4. Timely therapy initiation
5. Structured follow-up
6. Real-world evidence collection

Without these, value cannot be demonstrated.



11 years ago ...

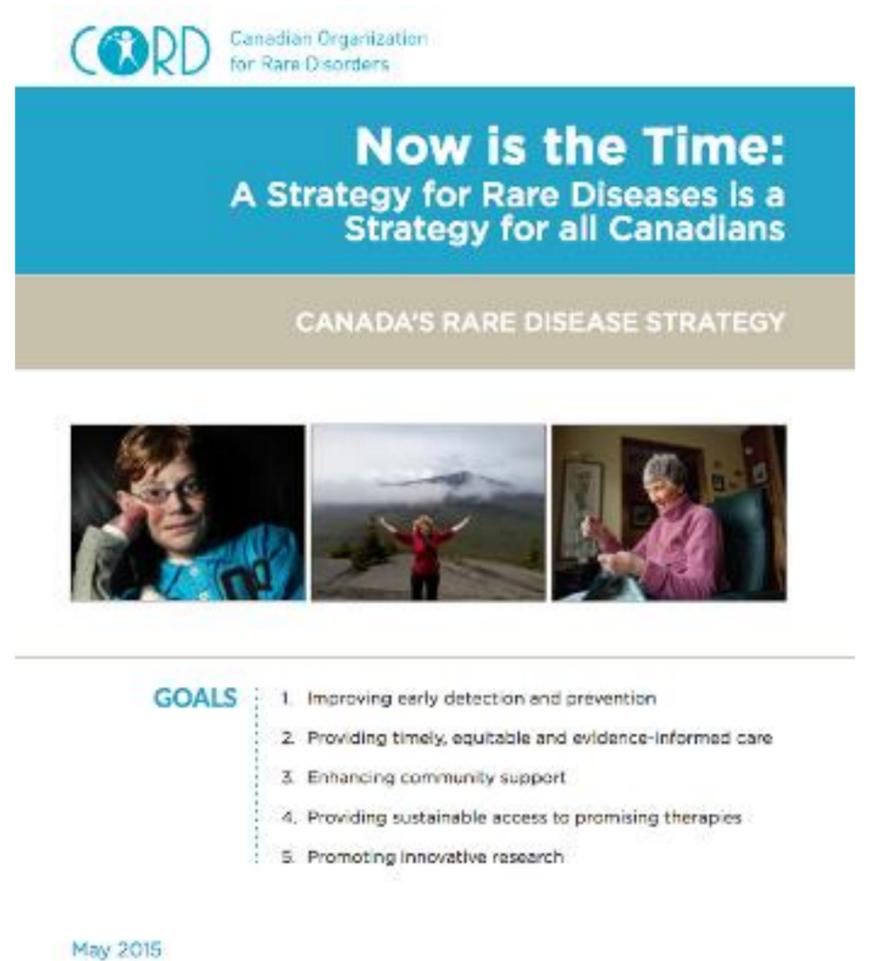
CORD Proposed Canada's Rare Disease Strategy: 5 Key Pillars

1. Improving early detection and prevention
2. Providing timely, equitable and evidence-informed care
3. Enhancing community support
4. Providing sustainable access to promising therapies
5. Promoting innovative research

Today ...

Phase 1 if RDDS has made strong investment to addressing Pillar 4.

Phase 2 invest in Pillars 1–3 and 5 to assure outcomes for patients and society.



Evidence & Follow-Up: The Missing Link

The Strategy commits to:

- “Collect and use evidence”
- Support sustainability

We must provide:

- Transparent national framework for outcome tracking
- Public provincial reporting on utilization and equity
- Increased visibility on real-world effectiveness
- Only with follow-up will we have proof of value.



Phase 2: From Drug Strategy to Rare Disease System Strategy

To fulfill federal objectives on Phase 1 of RDDS and lay framework for Phase 2:

- Public reporting on provincial allocation & uptake
- National diagnosis acceleration initiatives
- Standardized referral pathways & centres of expertise
- Structured real-world data collection
- Integration of supportive services & financial protections
- Framework for ultra-rare/no-approved-therapy conditions

Canadians Are Still Advocating for...

Rare Disease Drugs Today





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