Rare Disease Drug Access in Canada: Current Snapshot and Timelines

Canadian Organization for Rare Disorders
Rare Disease Day Summit 2025

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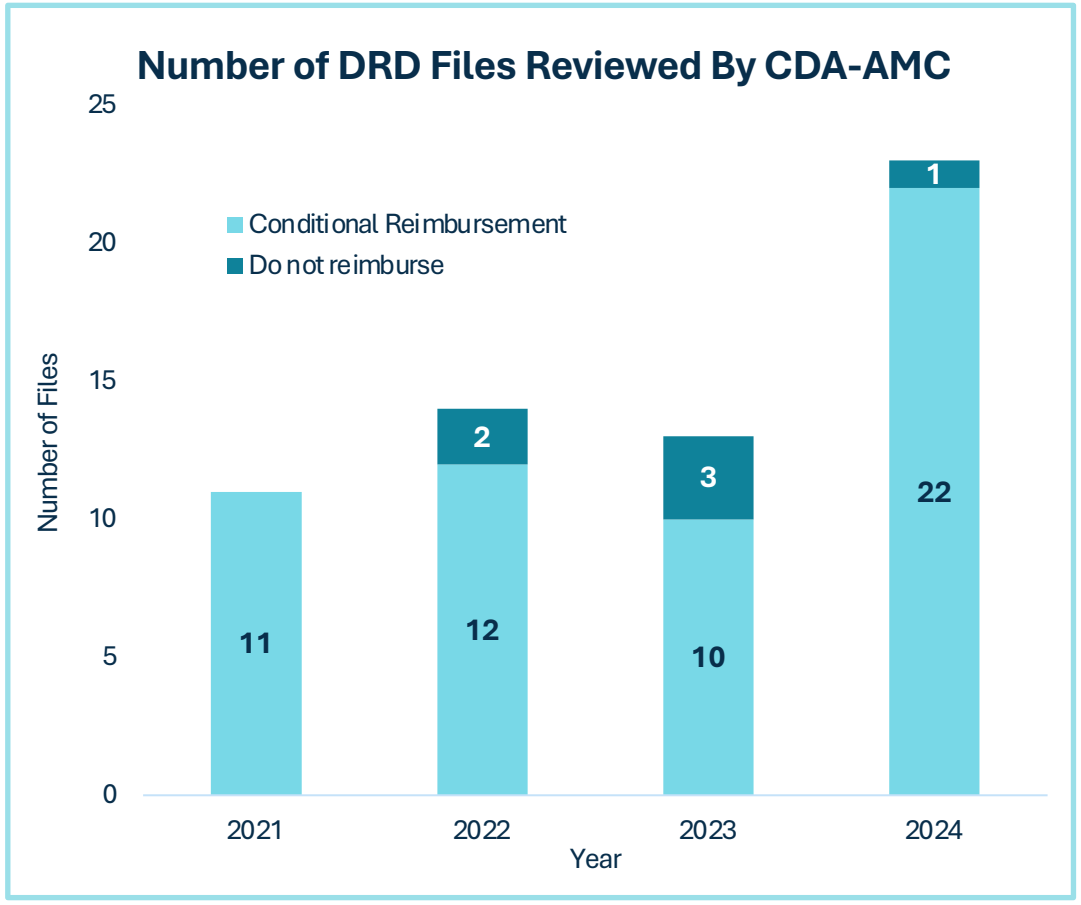
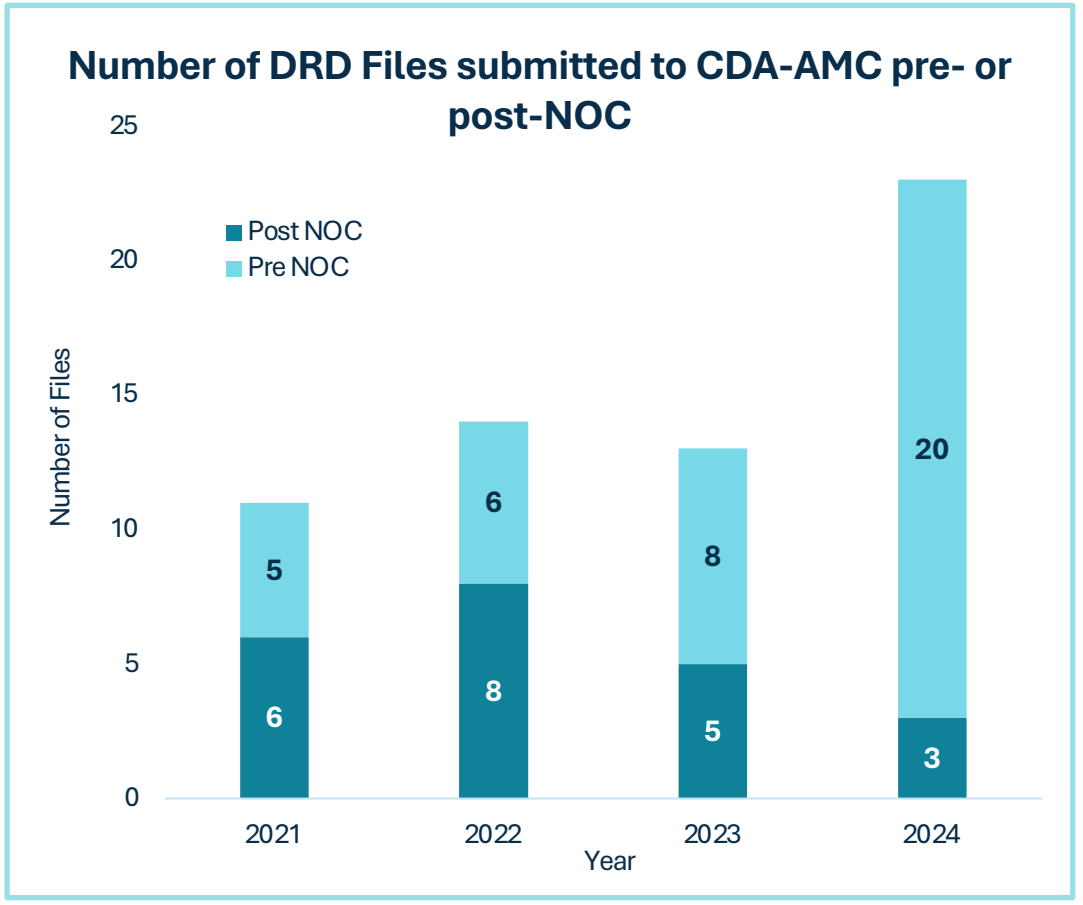
Of the 61 non-oncology drugs for rare diseases (DRDs) reviewed by CDA-AMC over the last 4 years 90% received positive CDA-AMC recommendations & 64% were submitted pre-NOC

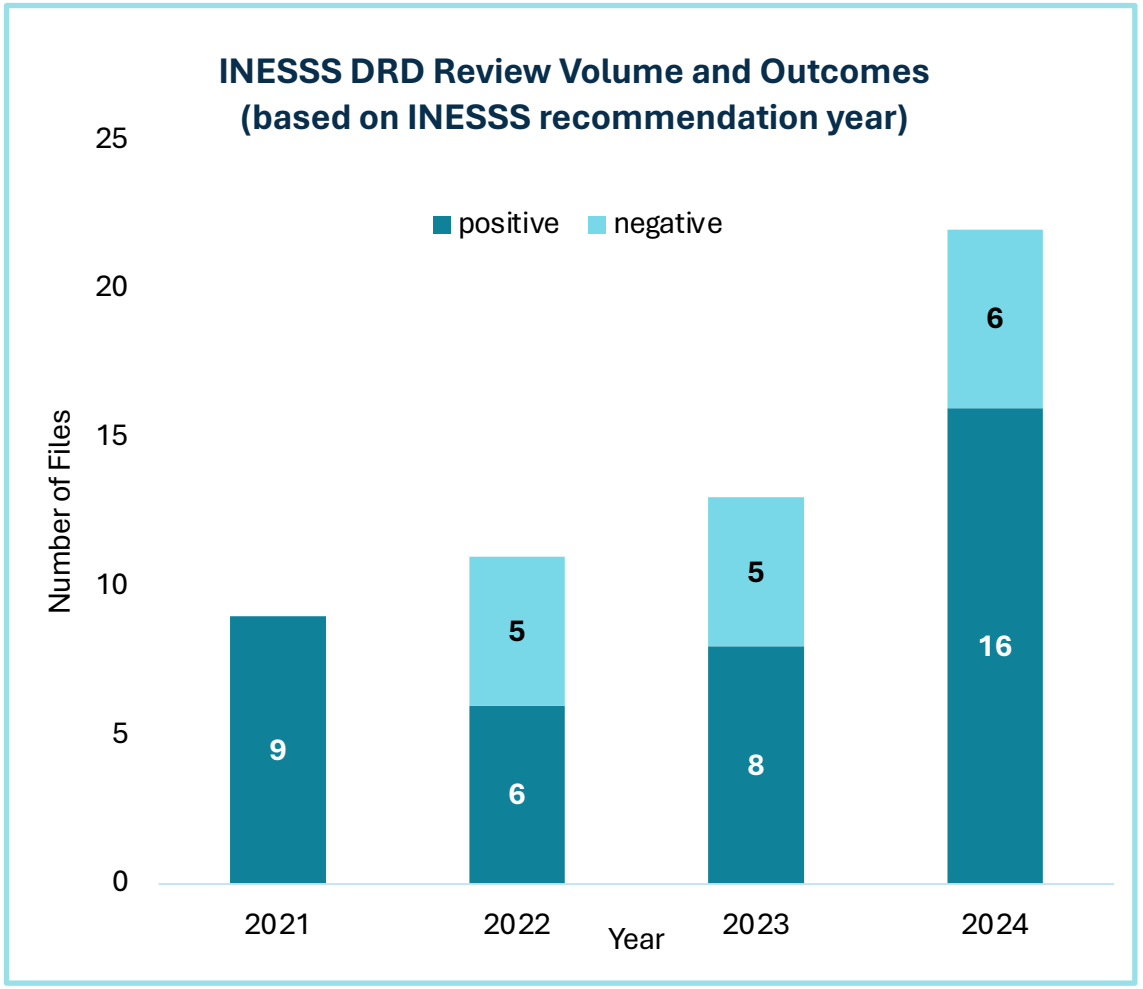
Pre-2018, less than 70% of DRDs received positive CDA-AMC recommendations.





Note: This analysis is based on the methodology used for MORSE's CRaFT DRD Sub-report; oncology and blood products are not included.

INESSS has reviewed 55 non-oncology DRDs over the last 4 years, with 71% receiving a 'positive' therapeutic value assessment

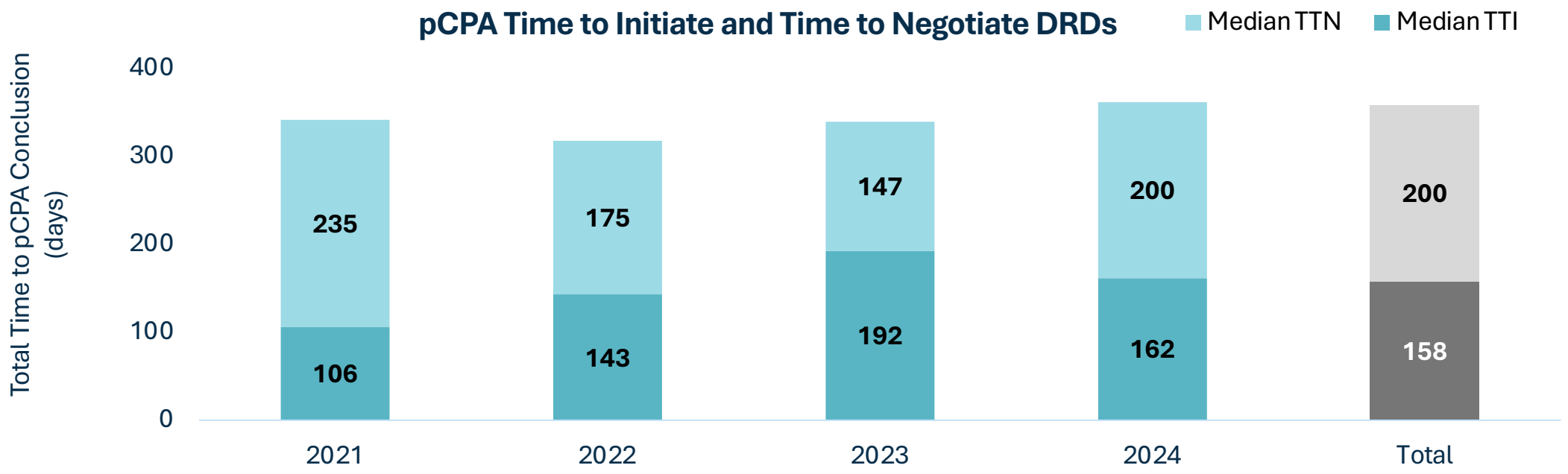


In the past 4 years, for the 61 files that reviewed by **CDA-AMC**, 45 drugs (74%) had the same evaluation results from CDA-AMC and INESSS.

Number of Files	CDA-AMC	INESSS
41	Positive	Positive
12	Positive	Negative
2	Positive	Under Review
2	Negative	Positive
4	Negative	Negative

Note: This analysis is based on the methodology used for MORSE's CRaFT DRD Sub-report; oncology and blood products are not included.

Negotiations for DRDs have seen large variability in timeliness both year over year as well as within year. If we look at best cases across the last 4 years, we see faster timelines are achievable.



Year	2021	2022	2023	2024	Grand Total
#Files Initiated	12	11	8	20	51
#Files Completed	4	10	10	7	31

TTI = Time to Initiate (days); TTN = Time to Negotiate (days)

Given the variability in timing for DRDs, we looked more closely at the outliers, those with faster & slower timelines

Fast:

- Prioritization for initiation does happen with the fastest 10% of files being initiated in less than 20 days, including one being initiated ahead of the CDA-AMC final recommendation
- Opportunity for earlier negotiation of subsequent indications

Slow:

- Availability of other therapies in rare diseases may be impacting priority
- More complex agreements can take more time initially but could pave way for other products or indications to come through more quickly
- Drugs with complex implementation often take longer to negotiate, and drugs with discordant HTA recommendations often take longer to engage

Source: MORSE market access database & CRaFT 2025 DRD Sub-report

The impact of the National Strategy for Drugs for Rare Diseases on access timelines is not yet clear, but inconsistency across jurisdictions is apparent

Up to $1.4 billion in funding available to provinces and territories through 3-year agreements, to help them provide better coverage and access to:

- **Elected new drugs for rare diseases on the common list**
- **Other new and existing drugs for rare diseases**
- **Screening and diagnostics services**

Province	Elected drugs on the common list, by province or territory					
	Poteligeo	Oxlumo	Epkinly	Welireg	Yescarta	Koselugo
BC	Yes	Yes	Yes	Yes	Yes	Yes
AB	Yes	Yes	Yes	Yes	No	Yes
SK	Yes	Yes	Yes	No	No	No
MB	Yes	Yes	Yes	Yes	Yes	No
ON	Yes	Yes	Yes	Yes	Yes	No
QC	Yes	Yes	Yes	Yes	Yes	Yes
NB	Yes	Yes	Yes	Yes	No	No
PE	Yes	No	Yes	No	No	Yes
NS	Yes	Yes	Yes	Yes	Yes	Yes
NL	Yes	Yes	Yes	Yes	Yes	No
YT	No	No	No	No	Yes	No
NT	Yes	No	No	No	No	No
NU	Yes	No	Yes	Yes	Yes	No

While challenges persist, there are feasible opportunities for improving access and reaching common objectives

Lean on the stated goals and aims of the National Strategy for Drugs for Rare Diseases to support the development of a risk mitigation framework that streamlines review, negotiation and funding for high need, complex drugs, starting early in the process & including multiple stakeholders

The stated goal of the Common List of new drugs is to ensure that the National Strategy for Drugs for Rare Diseases delivers the most possible benefits to all patients with rare diseases.

- Not all DRDs will be on the Common List, but access to DRDs overall could benefit from the eventual development of a common framework

Some objectives across stakeholders

- Increase transparency & improve dialogue amongst all stakeholders to find ways to manage uncertainty
- Increase speed to access for innovative drugs important to patients
- Create a menu of agreement structures recognizing uniqueness of DRDs/complex drugs
- Reduce administrative burden for drug plans, pCPA, manufacturers, and patients

Alignment with work already underway

- National DRD Strategy
  - collecting data
  - making informed decisions
  - expanding coverage of drugs for rare diseases
  - improving screening and diagnostics for rare diseases
  - exploring how to improve availability of drugs for rare diseases
- HTIP
- CDA-AMC/pCPA: TLR/pTAP
- Towwers Institute
- 20Sense Led-Work

Thank You!

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