MORSE CONSULTING | DECIPHERING MARKET ACCESS

Canadian Pathway to Rare Disease Drug Access Snapshot on where we are with time to access

Canadian Organization for Rare Disorders
Rare Disease Day Summit 2024

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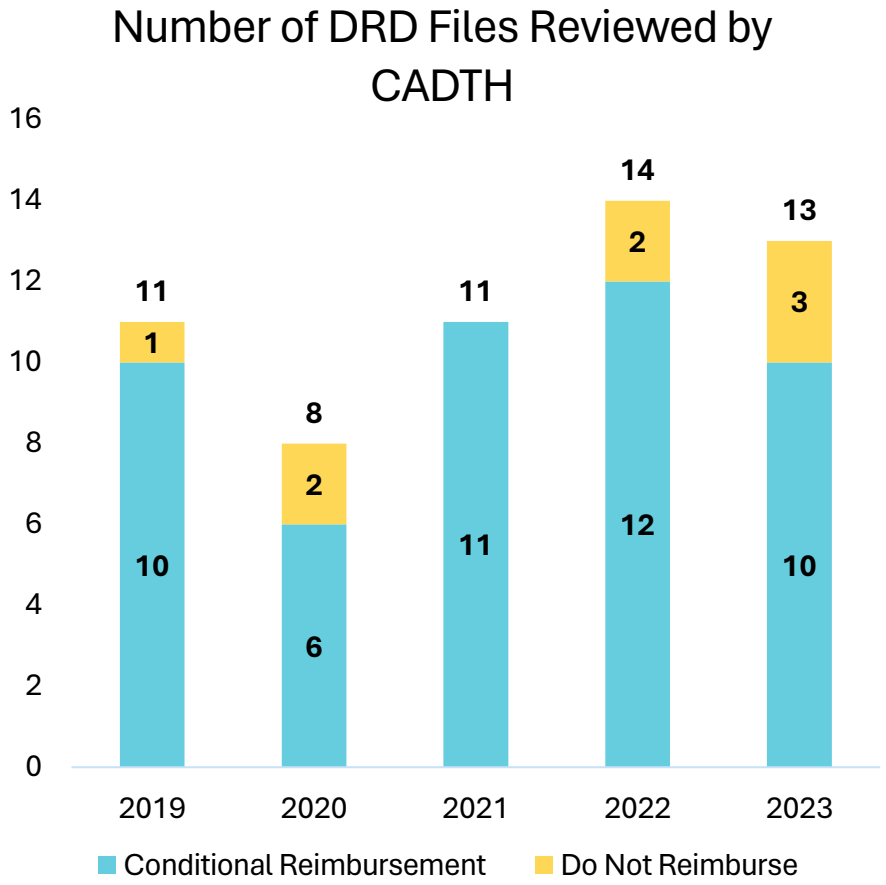
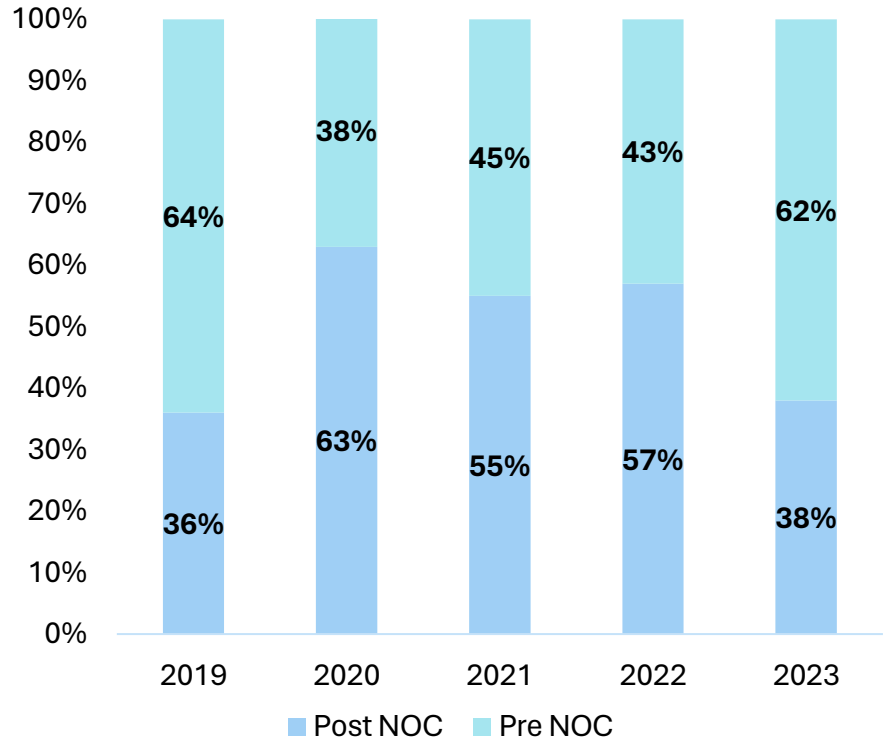
Of the 57 non-oncology drugs for Rare Diseases (DRDs) reviewed by CADTH over the last 5 years 86% received positive CADTH recommendations & ~50% were submitted pre-NOC

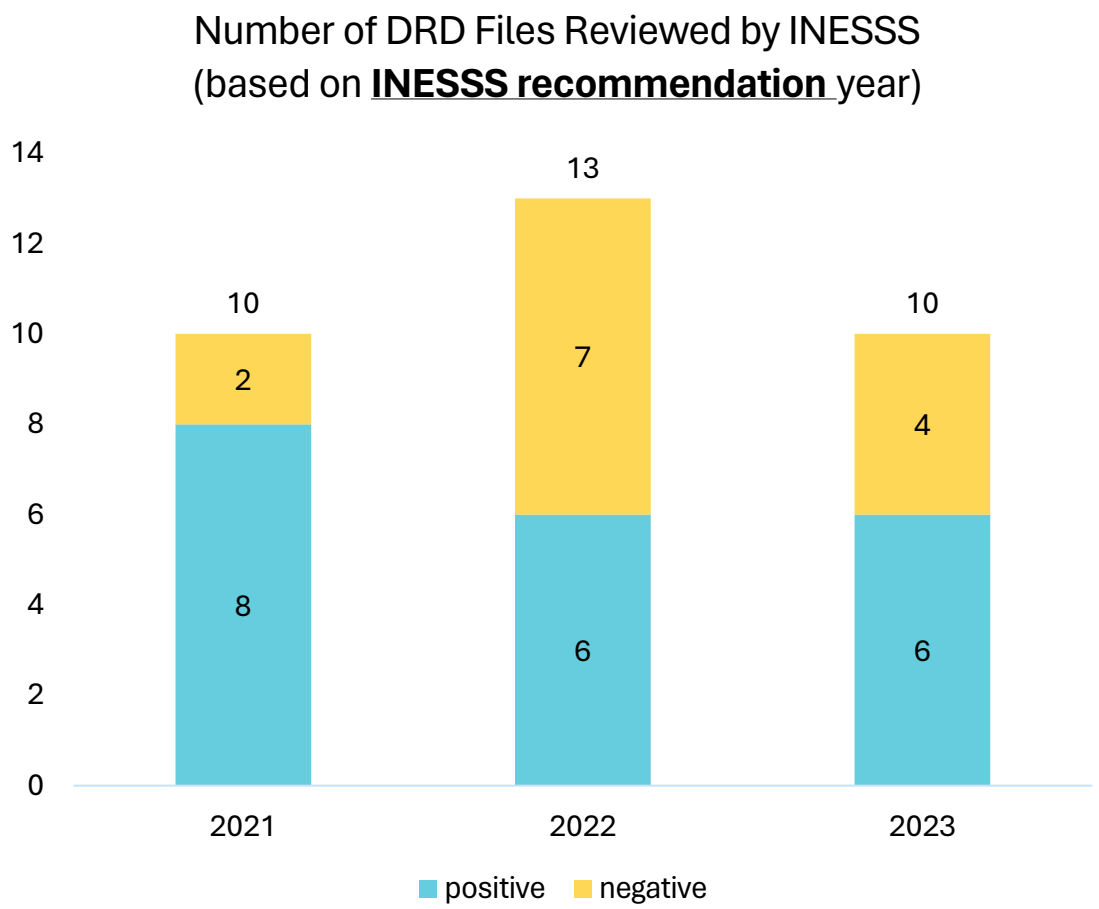
Pre-2018, less than 70% of DRDs received positive CADTH recommendations.





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 33 non-oncology Drugs for Rare Diseases (DRDs) over the last 3 years, with 61% receiving a 'positive' therapeutic value assessment

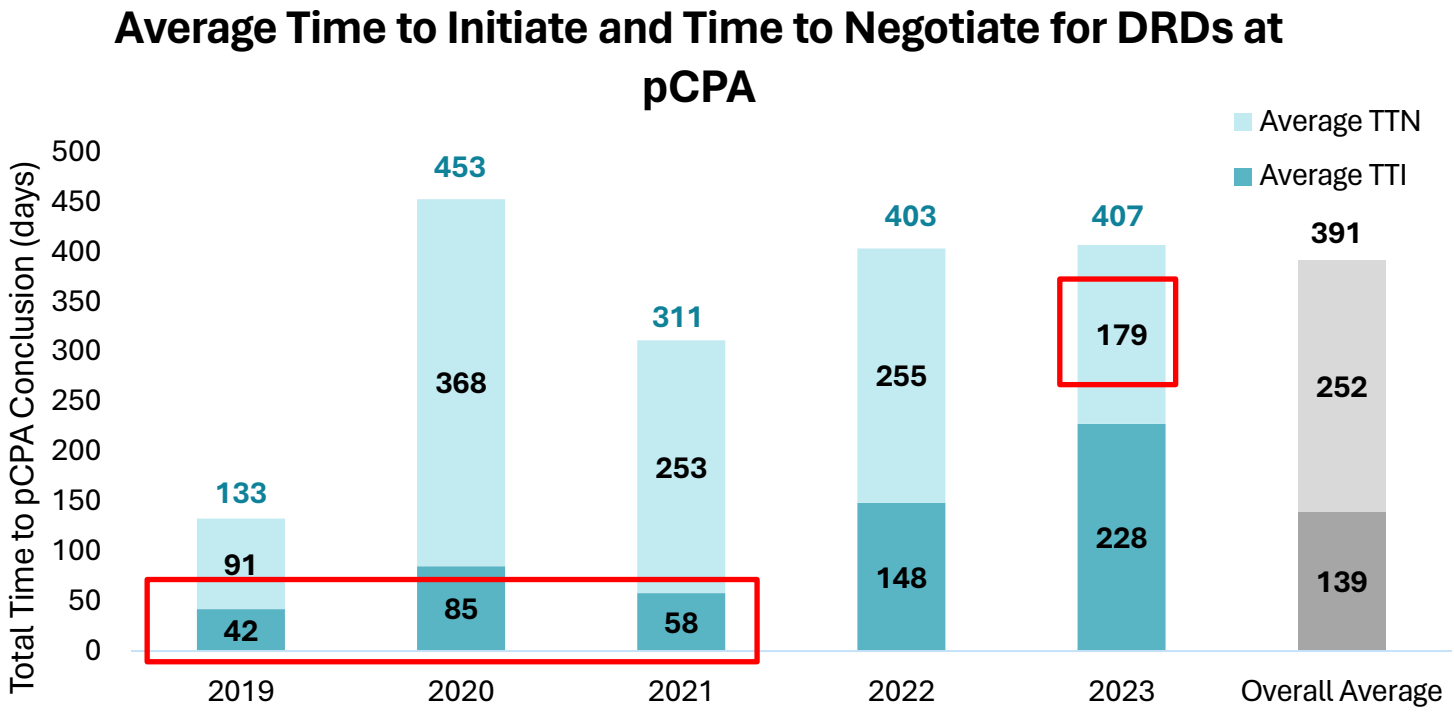


In the past 3 years, for the 38 files that reviewed by **CADTH**, 25 drugs (65%) had the same evaluation results from CADTH and INESSS. There is relatively high discordance between DRD HTA recommendations.

Number of Files	CADTH	INESSS
22	Positive	Positive
9	Positive	Negative
1	Positive	Not submitted
2	Positive	Under Review
1	Negative	Positive
3	Negative	Negative

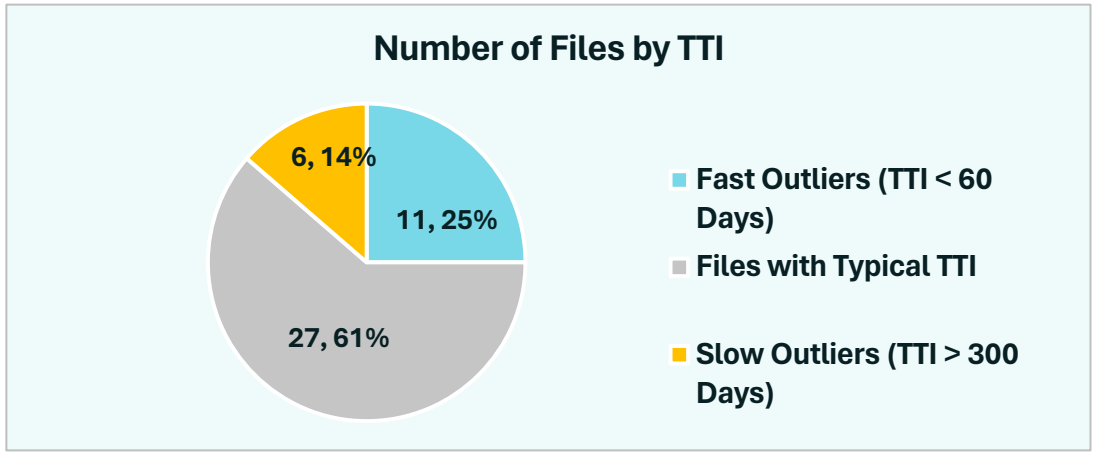
Note: This analysis is based on the methodology used for MORSE's CRaFT DRD Sub-report; oncology and blood products are not included.

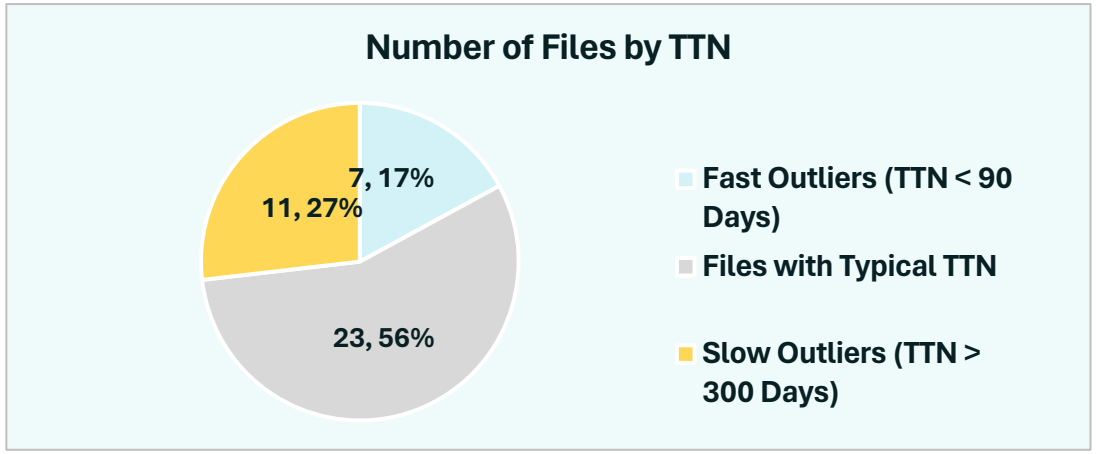
Negotiations for DRDs have seen large variability in timeliness both year over year as well as within year. If we look at best cases across the last 5 years, we see faster timelines are achievable



Year	2019	2020	2021	2022	2023	Grand Total
#Files Initiated	3	9	4	15	11	42
#Files Negotiated	4	12	6	15	11	48

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 ¼ of files initiating in < 2 months (3 pre-CADTH 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

Source: MORSE market access database;
TTI = Time to Initiate, calculated as the # of days between final CADTH recommendation and pCPA engagement letter;
TTN = Time to Negotiate, calculated as the # of days between pCPA engagement letter and LOI or Close Letter
DRD: Drugs for Rare Diseases, oncology and blood products are not included
CRaFT: Canadian Reimbursement and Forecasting Timelines Report

We all recognize there are real challenges, but there are also real opportunities for progress that are feasible & achievable

Develop a risk mitigation framework to support easier negotiation & listings for high need complex drugs that is considered early in the process & includes multiple stakeholders

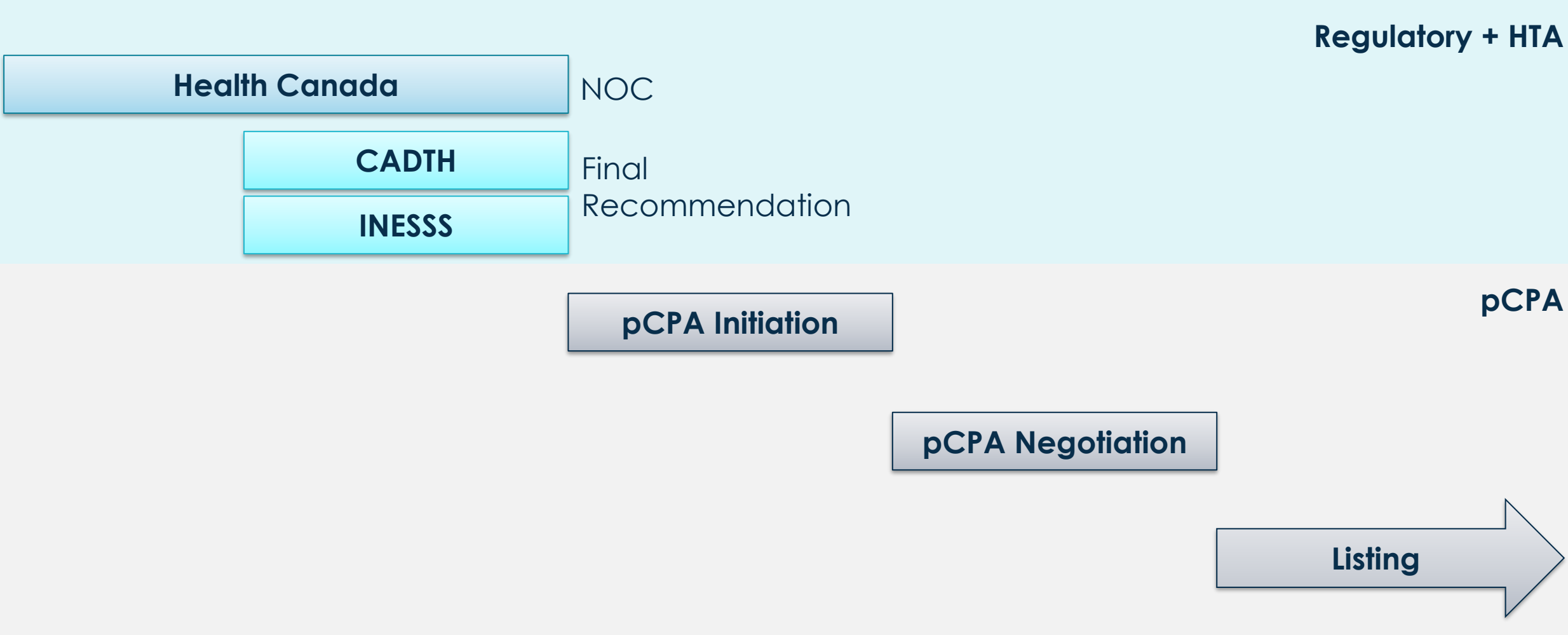
Objectives (not exhaustive):

- 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

Aligns with work underway by multiple parties – some examples:

- National DRD Strategy
- HTIP
- CADTH/pCPA: TLR/pTAP
- Towwers Showcase Project
- 20Sense Led-Work

Current State



Proposed Future State

Is this Future State Feasible?

- On average, 11 non-oncology DRDs go through CADTH each year (Range: 8-14)
- More than 90% of DRDs will achieve a positive recommendation from either CADTH or INESSS, requiring a pCPA negotiation
- Potential funding opportunities to support this early engagement
- Increasing capacity & resource at pCPA to potentially support more innovative pathways
- Learnings from Innovative Licensing & Access Pathway in UK

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Thank you!


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