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Innovative Pathways to Reimbursement

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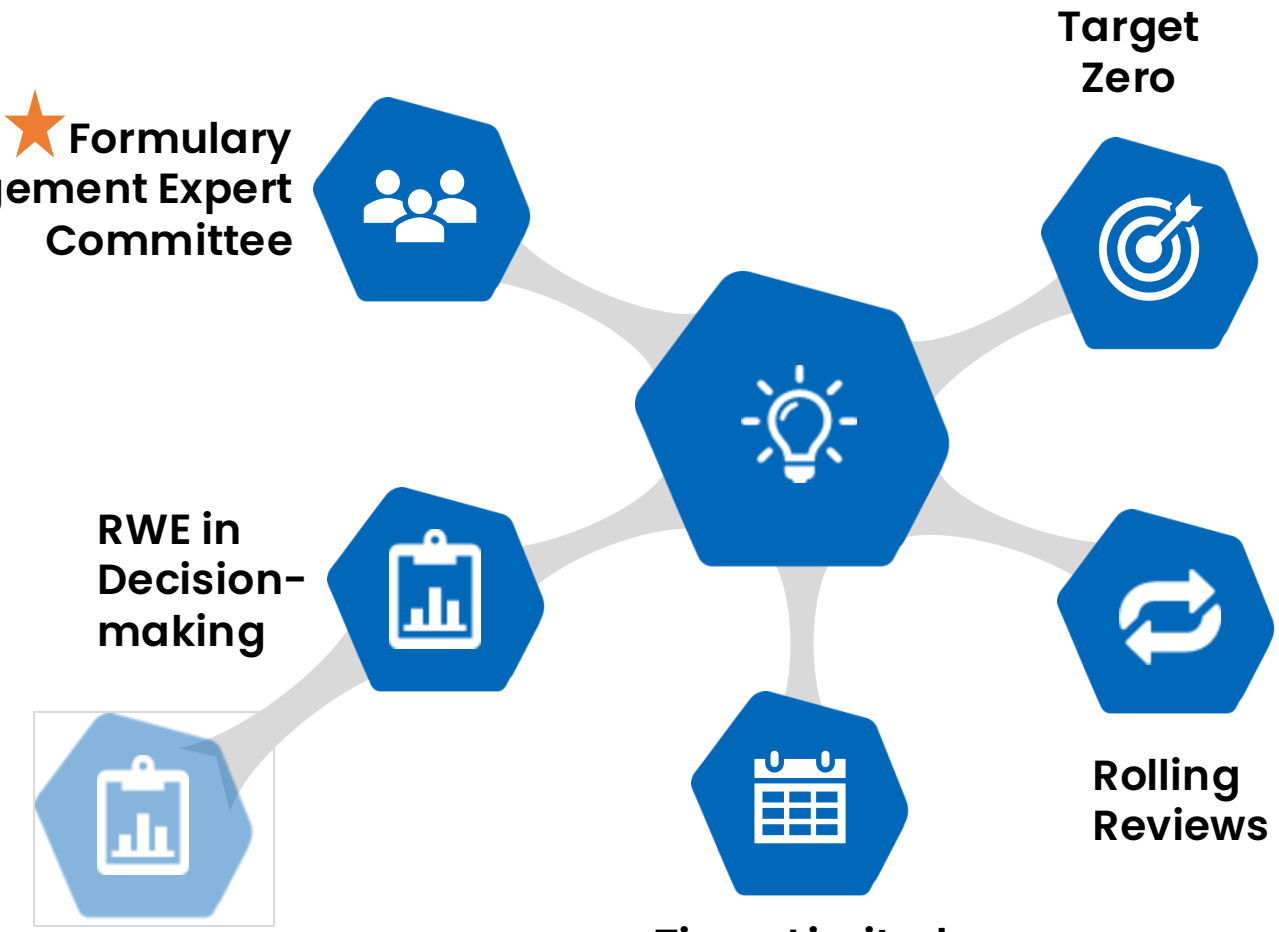
Disclosure

- The organization is funded by contributions from the Canadian federal, provincial, and territorial ministries of health.
- We receive application fees from the pharmaceutical industry for:
    - Our Reimbursement Review processes, including those used for:
        - oncology drugs
        - non-oncology drugs
        - plasma protein and related products reviewed through the interim process
    - Scientific Advice

Innovative Pathways to Reimbursement



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Time-Limited Recommendations

Time-Limited Recommendations

What are Time-Limited Recommendations (TLR)?

- a recommendation to publicly fund a drug for a period of time on the condition that:
  - Sponsor conducts one or more clinical studies that addresses uncertainty; AND
  - CDA-AMC will conduct a reassessment of the additional evidence.
- Reassessment will lead to a final reimbursement recommendation.

Approach

- support timely access to effective new therapies

Time-Limited Recommendations

Initial Assessment Criteria

1. **Regulatory status:** Drug reviewed through HC's NOC/c policy; AND
2. **Reassessment commitment:** Sponsor commits to filing reassessment in accordance with CDA-AMC TLR procedures; AND.
3. **Evidence generation plans:**

- A phase III clinical trial is planned and/or being conducted in a patient population that is reflective of the indication being reviewed by CDA-AMC; and
- the study completion date will not exceed 3 years from the target expert committee meeting date.
- The phase III study must be conducted in the same patient population as the indication under review by CDA-AMC (e.g., same line of therapy) using the same intervention being reviewed by CDA-AMC (e.g., the same dosage regimen specified in the product monograph).
- Study completion refers to the target date that will be publicly communicated through clinicaltrials.gov

Time-Limited Recommendations

Initial Recommendation:

- Ultimately up to the expert committee to determine whether a TLR will be issued
- A TLR will include a TLR-specific reimbursement condition

If a TLR is issued:

- Status updates on evidence requirements in the CDA-AMC recommendation will be requested twice a year
- Proactively inform CDA-AMC of any updates to the conduct of the phase III trial

Reassessment deliberation:

- Recommendation to remove TLR condition only (with or without revisions to other conditions)
- Recommendation to not reimburse

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RWE in Decision-Making

RWE in Decision-Making

What is the role of RWE in CDA-AMC Reimbursement Reviews?

- **Initial submissions:** Focus should be on applying RWE to address important gaps in the pivotal trial evidence (e.g., populations and outcomes not studied).
- Encourage complete reporting of methods and results (e.g., in accordance with Guidance for Reporting Real-World Evidence)
- **Resubmissions:** RWE can be acceptable to address important evidentiary gaps raised by the expert committee (particularly in situations where clinical trials are not feasibility).

RWE in Decision-Making

When has RWE been used in Recommendations?

Trikafta for Cystic Fibrosis (6 years and older)

- Evidence Gap: Uncertainty on magnitude of clinical benefit for patients with normal function (excluded from clinical trials)
- RWE: 3 studies submitted to address gaps in pivotal trial evidence (HELIO, PROMISE, and data from US patient registry).

Lutathera for Gastroenteropancreatic neuroendocrine tumours

- Evidence Gap: No clinical trial data for patients with pNETs
- RWE: NETTER-R study submitted to address gap

RWE in Decision-Making

Time-Limited Reimbursement Recommendations

Can RWE be a part of a reassessment after a time-limited reimbursement recommendation has been issued?

- Currently, focus of reassessment is on data from phase III trial.
- Consideration may be given to RWE generated to address additional gaps in the evidence; however, this must be provided in addition to the phase III trial data.

Phase 1 Deliverables: Disease Based Registries & RWE

Building the foundation for improved generation and access to real-world data from rare disease-based registries.

Completed:

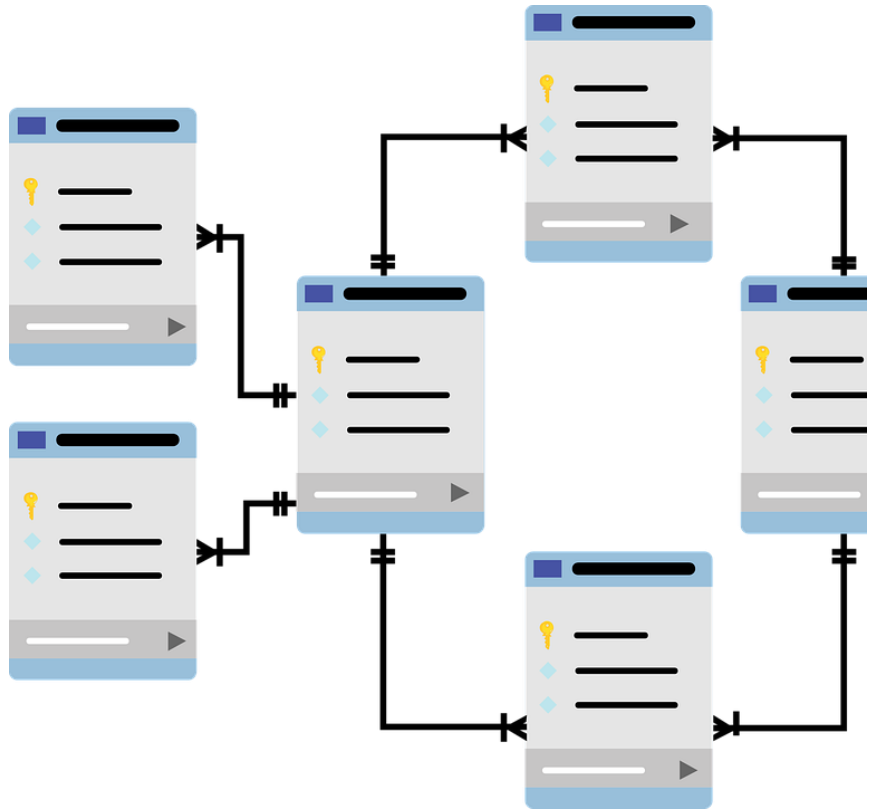
- Established an Inventory of Rare Disease Registries in Canada (March 2024)
- Initiated RFP process for grant funding of successful applicants (March 2024)
- Established an Advisory Group (April 2024)

In-progress:

- Produce Registry Standards and Guidance (anticipated September 2024)

Planned:

- Conduct 1 or 2 case studies to test the readiness and capabilities of existing rare disease registries in the context of health technology assessment (March 2025)



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Formulary Management Expert Committee

Formulary Management Expert Committee

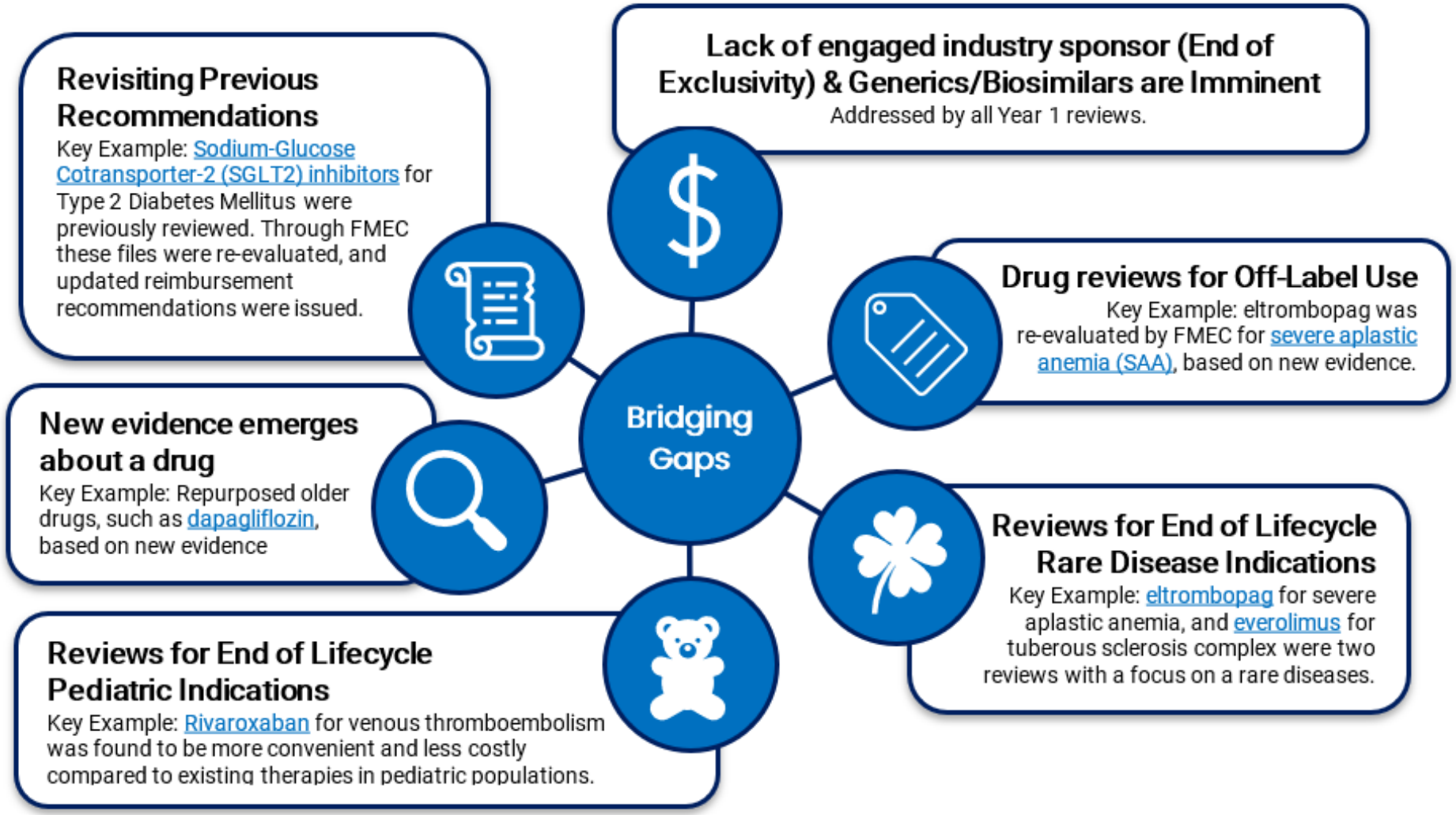
What is the Formulary Management Expert Committee (FMEC)?

- leverages opportunities to produce tailored reviews as requested by drug plans and to support the optimal utilization of drug benefits
- Recommendations are intended to increase the likelihood that reviews across the pharmaceutical life cycle will result in system impact

What are the benefits of FMEC?

- provide tools for payers to reimburse new indications for older drugs, or re-visit previous recommendations for older drugs – to increase treatment options

Gaps Addressed by FMEC



An Example of FMEC in Practice

2013

everolimus (Afinitor) for Renal angiomyolipoma associated with tuberous sclerosis complex (TSC)

- "do not reimburse"

2015

everolimus (Afinitor) for Subependymal giant cell astrocytoma associated with tuberous sclerosis complex

- "do not reimburse"

New Long-term Data

Innovate

Unleash the Value of Technology Across Its Lifespan

2023/24

Launch of the Formulary Management Expert Committee

2024

everolimus for Renal angiomyolipoma associated with tuberous sclerosis complex

- "Reimburse with clinical criteria and/or conditions"

2024

everolimus for Subependymal giant cell astrocytoma associated with tuberous sclerosis complex

- Expert committee deliberation Sept 19

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Target Zero

Target Zero

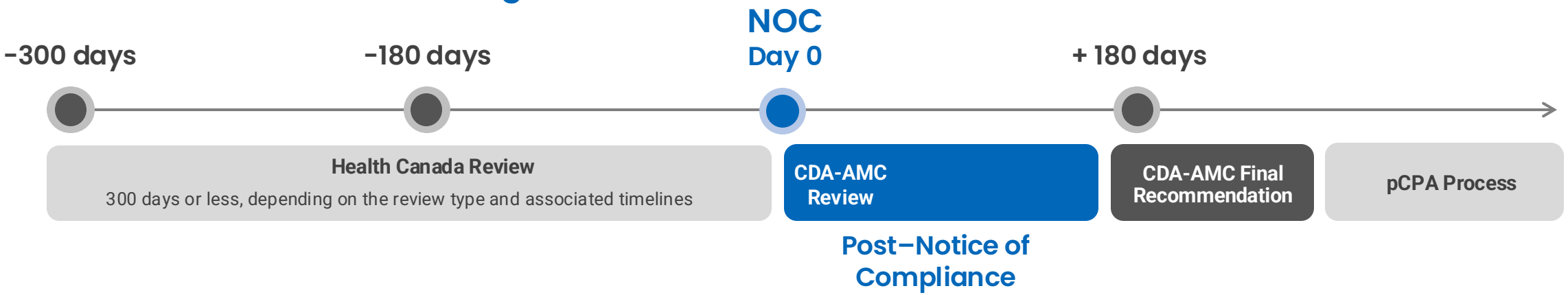
What is Target Zero?

- Initiative to target **zero days** from Health Canada's NOC date to CDA-AMC's draft reimbursement recommendation date by promoting the use of parallel submission processes.

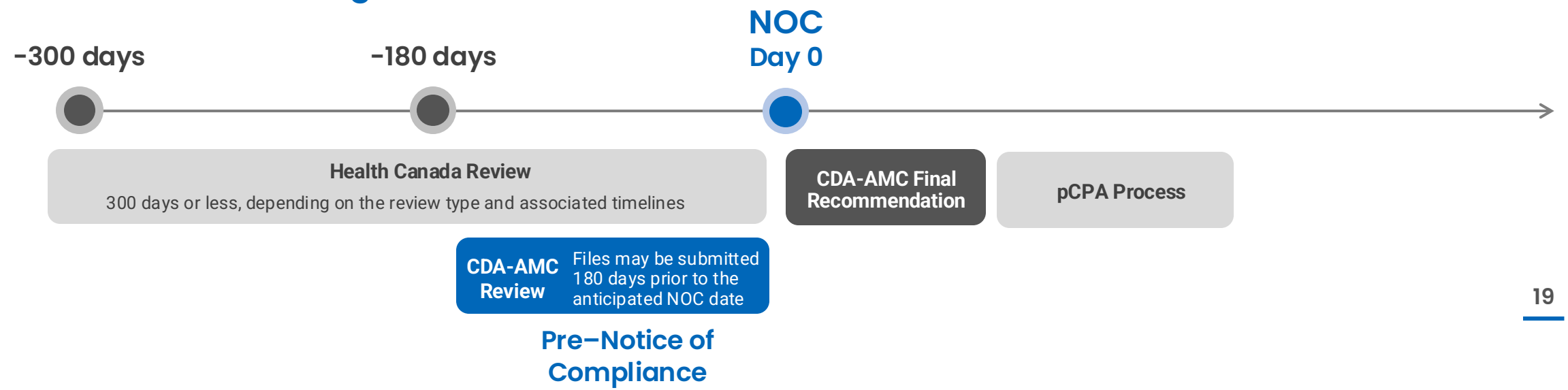
Does Target Zero work?

- Only files submitted 180 days prior to NOC can predictably achieve Target Zero

Reimbursement Review Timing



Parallel Review Timing



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Rolling Reviews

Rolling Reviews

What are Rolling Reviews?

- Sponsors can submit the required clinical and economic evidence as it is available rather than assembling all required documentation into a single application package
- Review timelines depends on the complexity of the economic submission and the timeline for filing the information

What are the benefits of Rolling Reviews?

- Our review can be initiated earlier without all the pieces being in place

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Where to from here?

Commitment to Developing Innovative Pathways to Reimbursement

June 2018

Aligned Reviews with Health Canada

Launch of the **Formulary Management Expert Committee**

June 2023

July 2023

Rolling Reviews for COVID-19 drugs

Launch of **Time-Limited Recommendations**

Sept 2023

October 2023

Target Zero launch

Rolling Reviews (pilot)

July 2024

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