CORD briefing for April 26, 2022 roundtable with Health Canada on Phase II of the National Strategy for Drugs for Rare Diseases

- **Date:** April 26, 2022, 1:00-3:00pm via Zoom
- **Participants:** Office of Michelle Boudreau

  CORD and other patient organizations

Welcome, introductions, and purpose of the meeting	5mins
Setting the stage presentation from Health Canada • Taking stock of what we heard to date, including areas where consensus is emerging • Draft framework for the national strategy for drugs for rare diseases • Key implementation considerations	15mins
Open discussion • Reactions to the draft framework for the national strategy • What can you as a stakeholder/partner contribute to advancing the strategy	35mins
Break	10mins
Implementation considerations – deep dive on specific issues • Collaborative decision-making and governance • Data systems • Involvement of stakeholders and the public in implementation	50mins
Closing remarks and next steps	5mins

- This two-hour roundtable is one of several being hosted by Health Canada in the same week. This roundtable is with pharma sector leaders, and the following day patient group leaders will be consulted. We do not know which other groups Health Canada intends to meet with (e.g., "health system partners" such as CADTH, pCPA Office, provinces; clinicians, etc.).
- There are two main elements of the roundtable based on the discussion paper: (a) the draft **strategy framework**; and (b) an **implementation approach** regarding three aspects: drug coverage; governance; and evidence generation infrastructure.
- The draft strategy framework in the Discussion Guide has some notable differences from the draft strategy that was shared in August 2021 (before the federal election). Since then, they have been conducting *ad hoc* bi-lateral meetings to discuss the rare disease drug strategy. This is the first multi-stakeholder consultation to consider an updated Discussion Guide or proposed approach since the first half of 2021.
- **Positives:** Appreciate the "what we heard" and the basic principles is still there, which is good.
- **Drawbacks of the approach as proposed:**
    - But the implementation and execution appears very far from happening. This seems way too far away from securing and disbursing the funding. It doesn't – in terms of execution – relate to the previous consultations.

  - The document is **vague**, with very few definitions or explanations of who will do what, when and how. "Ethical," "decision-makers," etc. – it's not clear.
  - What we read is that the government will support a **national formulary** for drugs of "common concern." Starting with the basic list – drugs of "common concern" – is very problematic. These medicines are already included and funded. We're really concerned about the medicines that aren't available now.
  - **The concept of equitable access** should be incorporated to address uncertainty and the problems that we have. The concept of equity involves fairness – which is giving everyone a shot to a better and longer life.
  - There's **no mention of speedier access / earlier access**, which are hallmarks of the UK strategy. Australia as well has a new MoU with developers to accelerate access and bring in new medicines to start patients on drug early. Even ICER in the US: basic principles for what we're trying to do: faster access to new medicines and security of supply. None of those aspects are in this document.
  - These are plans and **solutions that have nothing to do with the problem** that we're trying to answer. The OHIP+ debacle, for example, was a solution in search of a problem.
  - **Missing**: infrastructure, diagnostics, centres of excellence, managed access, etc.

- The concepts of **agility and adaptive** should be at the clinical level: if we've got good diagnostics, patient registries and good clinicians who are funded and empowered to support these patients, we can get these medicines into the clinic.

- Discussion questions for the **framework** include the following:

Questions (from Discussion Guide)	**Potential key messages and questions / considerations**
What are your initial reactions on the principles and pillars as described in the draft framework for the strategy?	• See above:
What are your thoughts on the potential activities proposed to support the strategy? Which activities would you prioritize?	• Build the system and invest in it accordingly. This is like asking what's most important for you in a house that you're building – it all comes together. • Invest in centres of excellence and then you can build out from there. This is disease-focused, not drug-focused. The NCE approach will empower clinicians and patients.
Where could you see yourself, your organization, or your industry contributing or playing a role to support and advance the draft framework for the national strategy?	• We'll support the draft framework when we see it as focused on solving for rare diseases as opposed to re-reviewing and developing a closed formulary for drugs. We have a 12 step process that proposes a holistic system. • Case study approaches could be used to model out how the strategy would be implemented in practice.

	• Surveys among our members / community asking them: does this strategy position Canada and patients for success?

- Health Canada also proposed an *iterative approach* to implementation and continuous learning and agility to evaluate, adapt, and improve. The goal is to address immediate and common pressures and yield several outputs to help shape future decisions and adaptations.
- They proposed specific activities under three themes in a "foundational phase": 1-Drug coverage, 2-Governance and 3-Evidence generation infrastructure.
- **Drug coverage**:
    - On a key issue, funding, the only language in this section is regarding "supporting coverage for select drugs of common concern." This initial list of drugs could form the basis to work toward a formulary for drugs for rare diseases.
    - NOTE: the example activities appear to draw on the pan-Canadian formulary initiative, including "Adopting common principles and developing a decision-making framework to assess and manage a formulary of Drugs for Rare Diseases"
    - On horizon scanning and planning, this may already be undertaken by other bodies (e.g., CADTH) and it's not clear who would do this for the RDDS (Health Canada?).
- **Governance**
    - A focus on consistency and a pre-condition is decision-making and advisory structures "to manage and improve the list of select drugs and evidence generation activities over time." The Discussion Guide recommends leveraging existing structures as much as possible.
    - Activities include "fit-for-purpose advisory committee(s) and working groups" (note industry is included in the list of stakeholders); exploring the feasibility of sustainable and innovative cost-sharing/risk-sharing models with multiple payers; and building relationships with international partners and networks and fostering information sharing among health system partners.
- **Evidence generation infrastructure**
    - Infrastructure for evidence generation to improve drug coverage decision-making over time and across the drug lifecycle; RWE, creation of adaptation of patient registries, data framework / data standards, data-sharing agreements; investments to support improved knowledge of rare diseases and drugs for rare diseases and patient outcomes.
    - Activities include a "data governance framework" a plan for a data system, **etc.**
- Discussion questions for the framework include the following:

Questions (from Discussion Guide)	**Potential key messages and questions / considerations**
How do we ensure collaborative decision-making that is inclusive of multiple perspectives?	• The real question should be how to ensure the decision-making is in the hands of the right stakeholders. You don't want to have collaborative decision-making on funding that's far from the clinic.
What types of advisory committees / working groups would be key for successful implementation, while ensuring efforts are not duplicated?	• Agree on the concept of leveraging existing infrastructures and organizations, however, some of these have failed to be inclusive and none involve drug developers (e.g., CADTH). • Other committees are already not fit for purpose. CDEC (CADTH) is for common drugs. • This is an opportunity to change that approach. • **Most importantly, and to re-emphasize, these review bodies should not be opining on specific medicines for inclusion on a national formulary – they should be overseeing the funding and support for clinical centres of excellence that would focus on improving health outcomes via access to RD treatments.**
What would be the first steps to planning a national data system / patient registry to support the implementation of the strategy?	• Are you contemplating a single patient registry or building on existing ones? Much of these efforts appear to be underway by other organizations (e.g., CADTH). • Focus on what's already there, and build on that. • Don't wait for perfection to move forward with funding.

Additional questions / comments to inform CORD's participation in Roundtable:

GENERAL COMMENT ON ACTIONS	• This strategy appears primarily focused on continuing to analyze and evaluate DRDs that are already over-evaluated and analyzed by multiple levels of review. As well, the framework does not mention accelerating access to DRDs for patients. • What are the plans regarding the funding commitment related to the strategy (e.g., the $500 million a year starting in 2022-23)? • How about metrics and specific outcomes that we want to accomplish? How will the vision of improving access to effective drugs and better health outcomes be measured and reported? What is meant by improving access more specifically (e.g., reducing timelines, broadening access and/or covering more patients)? What are the benchmarks for diagnosis, improved care and access to medicines (including time to funding)?