



Canadian Organization
for Rare Disorders

“SORRY TO INTERRUPT SUMMER HOLIDAYS” Webinar Series

Webinar 1: Aligning on Rare Drug Strategy,
PMPRB, and Federal Election
July 22, 2021





Canadian Organization
for Rare Disorders

“SORRY TO INTERRUPT SUMMER HOLIDAYS” Webinar Series

Webinar 2: Engaging Political Parties, Politicians,
and Policy Makers on Patient Needs
July 29, 2021



Welcome and Brief Overview

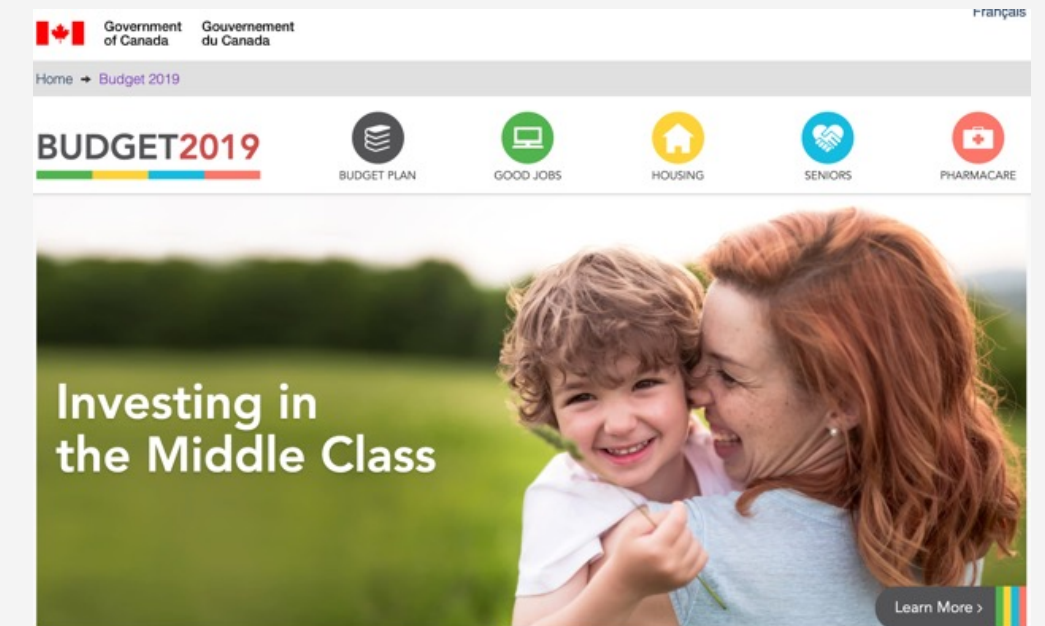
Durhane Wong-Rieger, CORD
Bill Dempster, 3Sixty Public Affairs

Three **BIG** Issues

- Reading the Tea Leaves on federal government's vision for Canada's "Expensive Drugs for Rare Diseases Strategy": What can we surmise from Health Canada's read-out on "What We Heard" during the consultations.
- Playing "Whack a Mole" with PMPRB: DOWN with implementation of new guidelines (3rd six-month hiatus); UP pops "out of the blue" pricing restrictions on grandfathered drugs.
- SURPRISE! (Not). Federal election this fall. How do we align to align all political parties and political candidates to support "rare disease" and "rare drug" strategy (and safeguard the \$500 million annual commitment)

Context and Opportunity

- Canada is one of the only developed countries that does not have a national plan for rare disease medicines
- 2019 federal budget includes a commitment to invest in a national strategy for rare disease treatments: **up to \$1 billion** over two years starting in 2022-23, with **up to \$500 million** per year afterwards
- 2020 fall economic statement and Speech from the Throne reaffirmed this commitment
- National support for better access to rare disease medicines has been building steadily since 2015 and CORD's launch of **Canada's Rare Disease Strategy**
- Both major national parties – Liberals / Conservatives included rare disease drug strategy in 2019 election platforms



Rare Disease Strategy - Five-point Action Plan

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 and
5. Promoting innovative research




CORD's 12-Steps to a National Rare Disease Framework

- **Patient empowerment:** Empower patient organizations and patient advocates as active full partners
- **Creation of a Canadian Rare Drug Agency:** independent, transparent, publicly accountable agency with responsibility for all aspects of the review of drugs for rare diseases, in coordination with Health Canada
- **Create R&D incentives:** Invest in Research and Development to support therapeutic product accessibility, monitoring, and evaluation. Build capacity for drug discovery, technological innovation, manufacturing and production, and (global) distribution.
- **Speed up access to treatment:** Ensure timely availability of new treatments by establishing a competitive and viable environment, including supportive mechanisms for clinical trials, early access programs, clinical site development, patient registries, and patient support programs



CORD's 12-Steps to a National Rare Disease Framework

- **Address regulatory barriers:** Ensure PMPRB guidelines do not exceed a “reasonable” threshold of fairness compared to comparable countries; roll back 2019 PMPRB regulatory changes by removing use of economic factors
- **Improve regulatory approvals process:** Ensure Health Canada continues to update its regulatory process to encourage clinical trial and new drug submissions for rare disease drugs
- **Ensure pathways for special cases:** For urgent need, timely access provided through Special Access Program (SAP) prior to Health Canada approval; create pathway through Early Access Programs.
- **Need for multiple funding options:** Multiple separate pathways based on population size, disease severity, unmet need, evidence uncertainty, potential therapeutic value, budget impact, annual unit price, and industry



CORD's 12-Steps to a National Rare Disease Framework

- **Leverage Managed Access Programs (MAPs):** For drugs receiving an NOC-C where there is uncertainty about the evidence at the time of approval but where the unmet needs and benefits outweigh the risks.
- **Facilitate concurrent Health Canada and HTA reviews:** Joint Health Canada and HTA application (where appropriate)
- **Support real-world evidence generation:** Be responsible for real-world monitoring, data collection, evaluating benefits, risks, and uncertainty
- **Enhance centres of clinical expertise:** Partner in developing Networked Centres of Expertise for specific rare diseases related to management of a therapy.

Canada Rare Drug Strategy: Pre-Election Activities Workstreams

Collaborative Canadian Network for Rare Diseases (CCN4RD)

- Nation-wide, cross-disease, cross-disciplinary, patient-inclusive network (physical and virtual) to support optimal use of Rare Disease Treatments, including locus of Real-World Data collection and management
- National hub within WHO-RDI Collaborative Global Network for Rare Diseases

Health Technology Assessment for Rare Disease Treatments

- Appropriate, consistent, transparent, and inclusive framework and methodology for assessing “place in therapy” of drugs and other treatments including cell and gene therapies
- Bridging uncertainties from clinical trials and real-world performance toward managed access/performance-based arrangements
- Inclusive of Real-World Evidence requirements and analysis

Canada Rare Drug Strategy: Pre-Election Activities Workstreams cont...

Patient data: Registries and Real-World Evidence

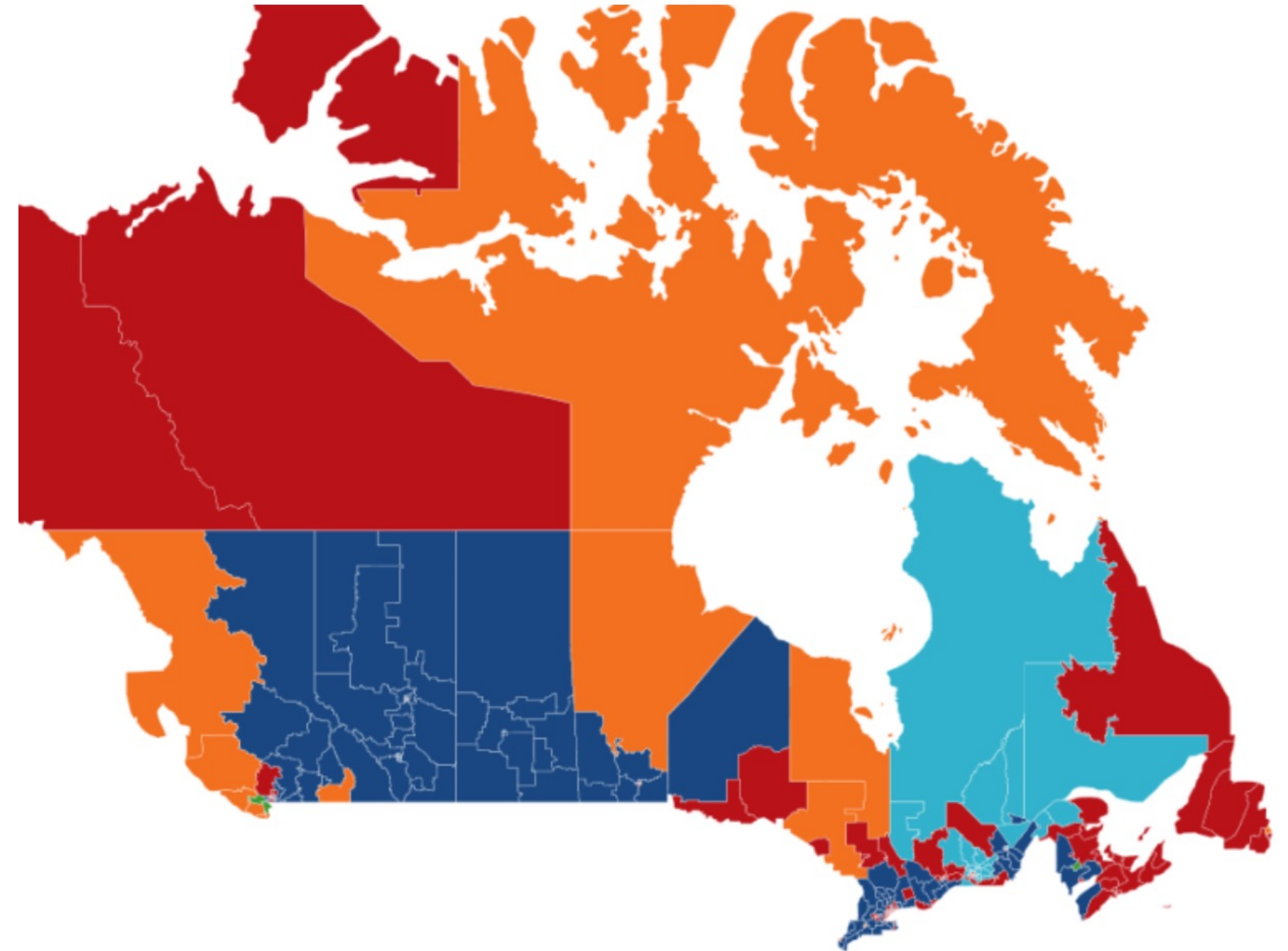
- Status of and best practices for patient registries in Canada and across jurisdictions; core and disease-specific elements; data-sharing and privacy
- Real-world evidence as foundational element for timely, monitored, and cost-effective usage of RD treatments

Value-based pricing of Rare Disease treatments

- Realistic picture of drug prices and costs in Canada across private and public drug plans and what this should look like
- Realistic assessment of Canadian drug prices within OECD countries and where we want to be
- How to strike the balance of timely access, value-based pricing, and sustainability of healthcare investment

Political situation

- Summer election call nearly a certainty despite PM's consistent denials
- Campaign teams, riding nominations, farewell speeches by exiting MPs, lawn signs ordered; COVID trends heading in the right direction
- Potential election timing: week of Aug 16/23 -> election Sept 27 or Oct 4
- Election calculus complicated & campaigns matter:
 - Liberals aim to make gains in BC and Ontario and hold current seats in Quebec
 - NDP / Jagmeet Singh's support rising which could split the left
 - Conservatives / O'Toole have higher "negatives" than other parties, but have not yet been in spotlight



Election issues / positions that could be relevant



**Justin
Trudeau**

**Erin
O'Toole**

**Yves-François
Blanchet**

**Jagmeet
Singh**

**Annamie
Paul**

- National Pharmacare
- Canada Drug Agency
- Rare Diseases Drug Strategy (\$500m/yr)

- Rare Diseases Treatments (\$500M/yr provinces-2019)
- Reform PMPRB

- Boost Canada Health Transfer

- “New Deal” that includes pharmacare

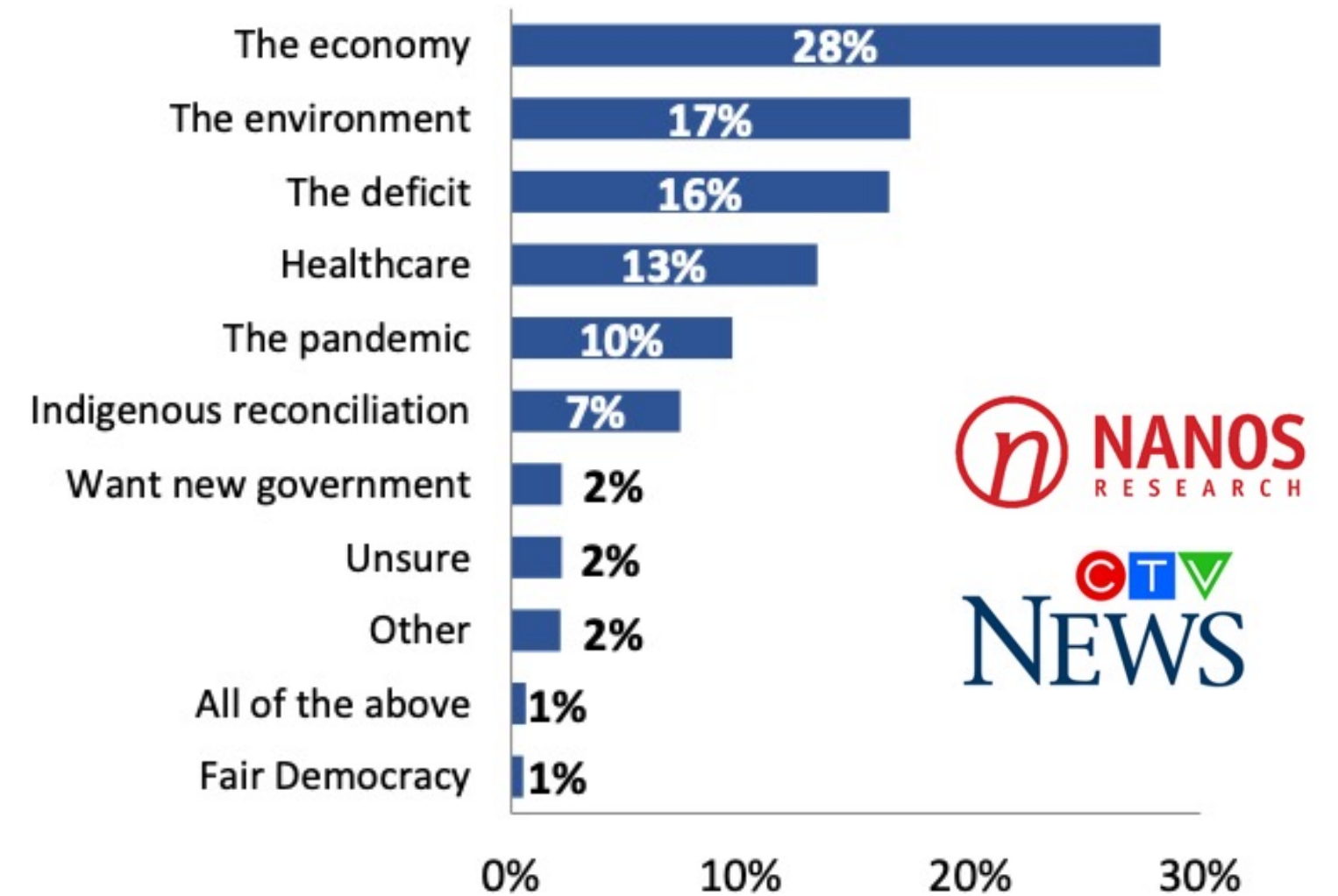
- National Pharmacare - basic formulary (2019)

What will the ballot box issue be?

- COVID pandemic response?
- Economic recovery?
- Deficits?
- Affordability (housing, gas, food, pharmacare, child care etc.)?
- Environment?



Most important policy that will influence vote



QUESTION: What will be the most important policy issue that will influence your vote if a federal election happens this coming Fall? [RANDOMIZE]

Source (graph): Nanos – National survey released July 2021. Nanos Research, RDD dual frame hybrid telephone and online random survey, June 30th to July 5th, 2021, n=1051, accurate 3.1 percentage points plus or minus, 19 times out of 20.

Challenge for today's town hall – how to translate CORD's key asks into a high-impact election plan?

Jumpstart Canada's Rare Disease Drug Policy (2021)

1. Patient Empowerment
2. Canadian Rare Drug Agency
3. R&D incentives
4. Speed access to treatment
5. Regulatory barriers
6. Regulatory approvals process
7. Pathways for special cases
8. Multiple funding options
9. Managed Access Programs
10. Concurrent HC/HTA reviews
11. Real world evidence
12. Centres of clinical expertise

Canada's Rare Disease Strategy (2015)

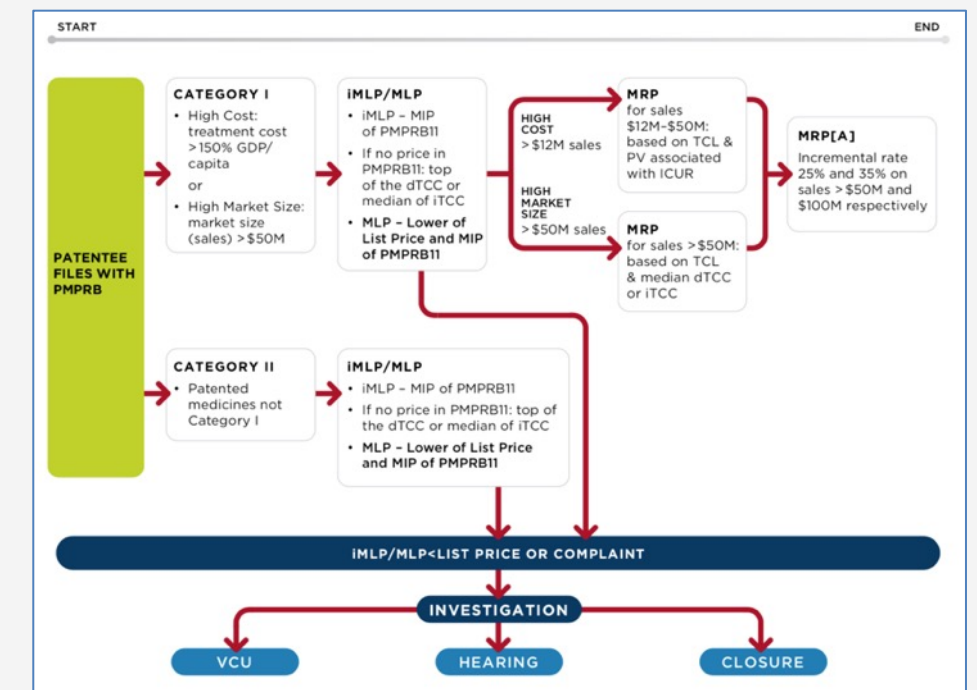
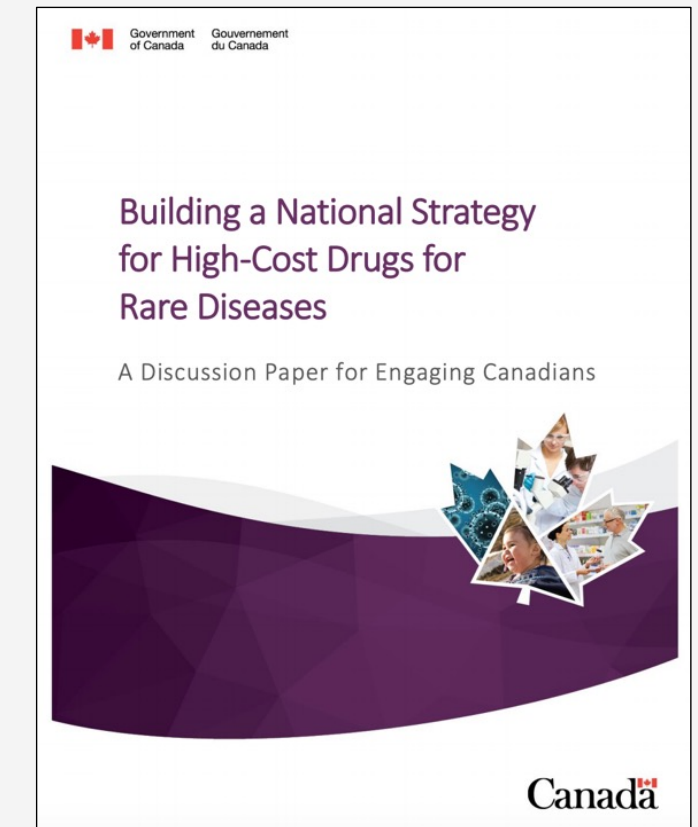
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What CORD can propose for the federal election?



Other key federal issues: update

- Rare Disease Drug Strategy “What we heard” report – expected imminently from Health Canada with more meetings to follow
- PMPRB changes delayed a 3rd time in June 2020, again in context of COVID pandemic:
 - New implementation date now January 1, 2022
 - After second delay, PMPRB launched communications / advocacy strategy; proposed reduced compliance timelines, then reversed
 - After this recent delay, PMPRB proposed adding new price reductions to reference country tests and speeding compliance timelines again – CONSULTATION OPEN UNTIL AUG 15/21



CORD's Mid-summer Super-panel and Town hall!

Panel

- **Peter Brenders**, BeiGene
- **Alexandra Chambers**, Novartis
- **Cathy Evanochko**, Tuberous Sclerosis Canada (TSCST)
- **Angela Genge**, The Neuro
- **Fred Horne**, 3Sixty Public Affairs
- **Leanne Ward**, CHEO

Moderators:

Durhane Wong-Rieger, CORD

Bill Dempster, 3Sixty Public Affairs