ONE-DAY LAUNCH OF A NATIONWIDE CONVERSATION

DECEMBER 16, 2020

11:00 AM – 5:00 PM ET

Designing the Blueprint for pan-Canadian Rare Drug Program

Why Now? Canadians have the Opportunity of a Lifetime to design the world's best Rare Drug Program directed to assure patients have access to today's and tomorrow's therapies. The Canadian government has committed $1 billion to set up Canada's Rare Disease Drug Strategy, with on-going investment of $500 million each year. The Rare Drug Program will be grounded in Canada's Rare Disease Strategy.

What is the Vision? The pan-Canadian Rare Drug Program will assure innovative and essential rare therapies are delivered to patients as soon as possible and consistent with Canadian principles of accessibility, comprehensiveness, universality, portability, and publicly accountable administration.

What is the Task? Stakeholders from all sectors will engage in a structured, informed conversation to design a blueprint for a pan-Canadian Rare Drug Program that will set the course for 10 months of cross-Canada dialogue to arrive at a plan for implementation by January 2022.

Final Agenda

11:00 am - 11:10 am	**Opening Remarks (10 min)** • Durhane Wong- Rieger, CORD
11:10 am - 11:40 am	**Patients Breaking Barriers (30 min)** • Cystic Fibrosis (Kalydeco) - Beth Vanstone • SMA (Spinraza) - Catherine Boivin • SMA (Zolgensma) - Brandon Vasey • aTTP - Mina Rajan • Cystic Fibrosis - Sharon Stepaniuk

CORD Canadian Organization for Rare Disorders

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11:40 am - 12:40 pm	**International Access Landscape: What works? What doesn't (60 min)** **Presentation:** Rare Disease Drug Pathways: Andre Vidal Pinhiero, Takeda Pharmaceutical Company Ltd **Discussants:** • Mike Drummond, University of York (UK) • Josie Godfrey, JG Zebra Consulting (former Associated Director NICE) • John Doyle, Pfizer • Rosalie Wyonch, C.D. Howe Institute • Sylvie Bouchard, INESSS
12:40 pm - 1:25 pm	**Current Pathways to Access: Case Examples (45 min)** **Case 1:** SMA Spinraza to Zolgensma - Alex MacKenzie, CHEO **Case 2:** Cystic Fibrosis Kalydeco, Orkambi, Trikafta - John Wallenburg, Cystic Fibrosis Canada **Case 3:** HPP and XLH Soft Bones - Leanne Ward, CHEO
1:25 pm - 1:45 pm	**Break (20 min)**
1:45 pm - 2:30 pm	**Current Canadian Status: What has changed? What has gotten better? What still needs to be better during phases of access process (45 min)** **Moderator:** Bill Dempster, 3Sixty Public Affairs **Topics:** ◦ Patient Engagement, Research, Regulatory, Value assessment, Managed access/Post-market, Pricing/Budget Impact **Panelists:** Regulatory: Anne Tomalin, Innomar Strategies CADTH/INESSS: Brent Fraser, CADTH Industry: Fred Little, Pfizer Access: Lindy Forte, Patient Access Solutions Patient Support: Sandra Anderson, Innomar Strategies Patient: Maureen Smith Policy: Fred Horne, Horne and Associates Research & Development: Étienne Richer, CHIR

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2:30 pm - 3:15 pm	**Building A Better Model (45 min)**
	
	Options:
	• Current with Adaptive procedures and flexibility for designated rare drugs
	• Current with Parallel Supplemental Process especially ultra-rare drugs
	• Stand-Alone Integrated Rare Drug Program for all rare drugs
	
	Issues:
	• Which rare drugs
	• Which drug programs
	• Funding/financing models
	• Governance
	
	Criteria/principles:
	• Canada Health Act: Accessibility, Comprehensiveness, Universality, Portability, Public administration
	• IOM: safety, effectiveness, efficiency, person centeredness, timeliness, and equity
	• (Hoskins) Pharmacare: distinct national process
	  ◦ Fair, consistent, equitable, timely, evidence-based access
	  ◦ Appropriate access despite clinical uncertainty; shows promise; potential outweighs risk
	  ◦ Follow up with real-world meaningful (individualized) indicators
	  ◦ Financial risk-sharing: performance-based agreements
	  ◦ Encourage innovation; catalyst to developing breakthrough medicines
	  ◦ National expert panel with strong patient representation; implement strategy, review individual cases, identify indicators of benefit
	  ◦ In place beginning January 1, 2022

3:15 pm - 3:30 pm	**Break and Consolidate Findings (15 min)**
3:30 pm - 3:45 pm	**Building Consensus: Feedback on Breakout Sessions on Points of Commonality and Divergence (15 min)**

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3:45 pm - 4:30 pm	**Rare Drugs: Addressing Unmet Patient Need, Investment, Revenue, Global Contribution: What does BEST look like? (45 min)** **Moderator:** Bill Dempster, 3Sixty Public Affairs 1. Vision Process: All 2. Panelist discussion ◦ MP, Calgary Shepard: Tom Kmiec ◦ Genome Canada: Ivana Cecic ◦ CADTH: Suzanne McGurn ◦ Access: George Wyatt, Innomar Strategies ◦ Clinicians/Researchers: Craig Campbell, Western University ◦ Policy: Fred Horne, Horne and Associates ◦ Patient Groups: (TBD) ◦ Research & Development: Rebecca Yu, Takeda
4:30 pm - 5:00 pm	**Conclusions and Next Steps (30 min)**

Canadian Organization for Rare Disorders