Filling the gap:

The case for a policy observatory to serve the Canadian rare disease policy ecosystem

CAPT 2024 Conference

October 21, 2024

Land Acknowledgement

Housekeeping

- Presentations will be made available on the CAPT web site after the conference.
- An evaluation survey will be sent out after the conference.
  - Feedback on this session and the whole event would be greatly appreciated.

Disclosures

- CAPT has received funding from Alexion AstraZeneca Rare Diseases Canada to support this panel session.
- Dr. Glennie is a consultant to a number of pharmaceutical companies with products in the rare disease space.
- The Institute of Health Economics (IHE) is a not-for-profit organization funded through grants and commissioned work from public and private sector partners.

Today’s Objective

- To provide an overview of the Canadian rare disease (RD) policy ecosystem
- To discuss the development of an initiative to identify and fill the policy development gaps to ensure informed decision making
- Perspectives: patients, clinicians, and policy makers

Speaker Introductions





- **Dr. Rebeccah Marsh -** Director, HTIP, Institute for Health Economics
- **Dr. Gail Ouellette** – RQMO and Co-chair, Health Canada Implementation Advisory Group (IAG) for the National Strategy for Drugs for Rare Diseases
- **Dr. Hernan Gonorazky** – Neuromuscular Program Coordinator, The Hospital for Sick Children
- **Marcel Saulnier** – Health policy consultant and former Associate ADM, Strategic Policy Branch, Health Canada
- **Dr. Judith Glennie** - J.L. Glennie Consulting Inc. (moderator)

Agenda:

1. Overview of the Canadian RD Ecosystem
2. Overview of Policy Observatory (PO) Concept
3. Respondents
   1. Patient perspective
   2. Clinician perspective
   3. Policy maker perspective
4. Audience Q&A

Overview of the Canadian RD Ecosystem

Dr. Judith Glennie

Background: Canadian Rare Disease Policy Context

Recent RD Initiatives

- Canadian Organization for Rare Disorders (CORD) Rare Disease Strategy (May 2015)
- Supplemental process for complex/specialized drugs (October 2018)
- pCPA Brief: Barriers to Access Treatment and Drugs for Canadians Affected by Rare Diseases and Disorders (December 7, 2018)
- Report of the Advisory Council on the Implementation of National Pharmacare (March, June 2019)
- Health Canada National Strategy for High-Cost Drugs for Rare Diseases (January 2021; on-going)
- CADTH RD HTA processes environmental scan (May 2021)
- Takeda RD international landscape report (December 2021)
- Quebec Policy for Rare Diseases (June 6, 2022)
- CADTH Multistakeholder Engagement Strategy to integrate RWE into decision-making about care for rare disease (June 2022)
- CADTH RWE for RD Best Brains Exchange Report (August 2022)
- CADTH RWE Guidance Working Group (August 2022)
- Takeda RD health system readiness report (August 2024)

Academic Groups

- Promoting Rare-Disease Innovations through Sustainable Mechanisms (PRISM) research initiative http://www.prismfive.org/
- UBC Emerging Research Cluster on Reimbursement and Pricing Policy for Drugs for Rare Diseases https://rare-diseases.ca/

Canada’s Federal Rare Disease Strategy (March 2023)

Improving access to drugs for rare diseases

CANADA.CA/HEALTH

Canadä

Jean-Yves Duclos
@jyduclos

Canada's first-ever National Strategy for Drugs for Rare Diseases is here! We are committing $1.5B over 3 years to improve access and affordability to the effective drugs that those living with #RareDiseases need.
➡ canada.ca/en/health-cana...

Improving Access to **Affordable** & **Effective** Drugs for **Rare Diseases**

The National Strategy for Drugs for Rare Diseases will support:

- Better screening & diagnostics
- More affordable medication
- Better access to medications
- The health of more Canadian

Quebec Rare Disease Plan of Action (May 1, 2023)

- **Aim:**
  - To improve the quality of life of people with rare diseases and their families by addressing their healthcare needs through coordinated efforts among healthcare professionals, patients, and their families.

- **Funding:** $17 million

- **3 pillars:**
  - Raising awareness and training among health professionals to improve their expertise in identifying and caring for patients with rare diseases
  - Facilitating and providing equitable access to diagnosis, care, and services for people with rare diseases
  - Promoting research and innovation to improve the understanding and treatment of rare diseases

- **Implementation timeline:** May 2023 - 2027

Health Canada and P&T gov

pCPA & PMPRB

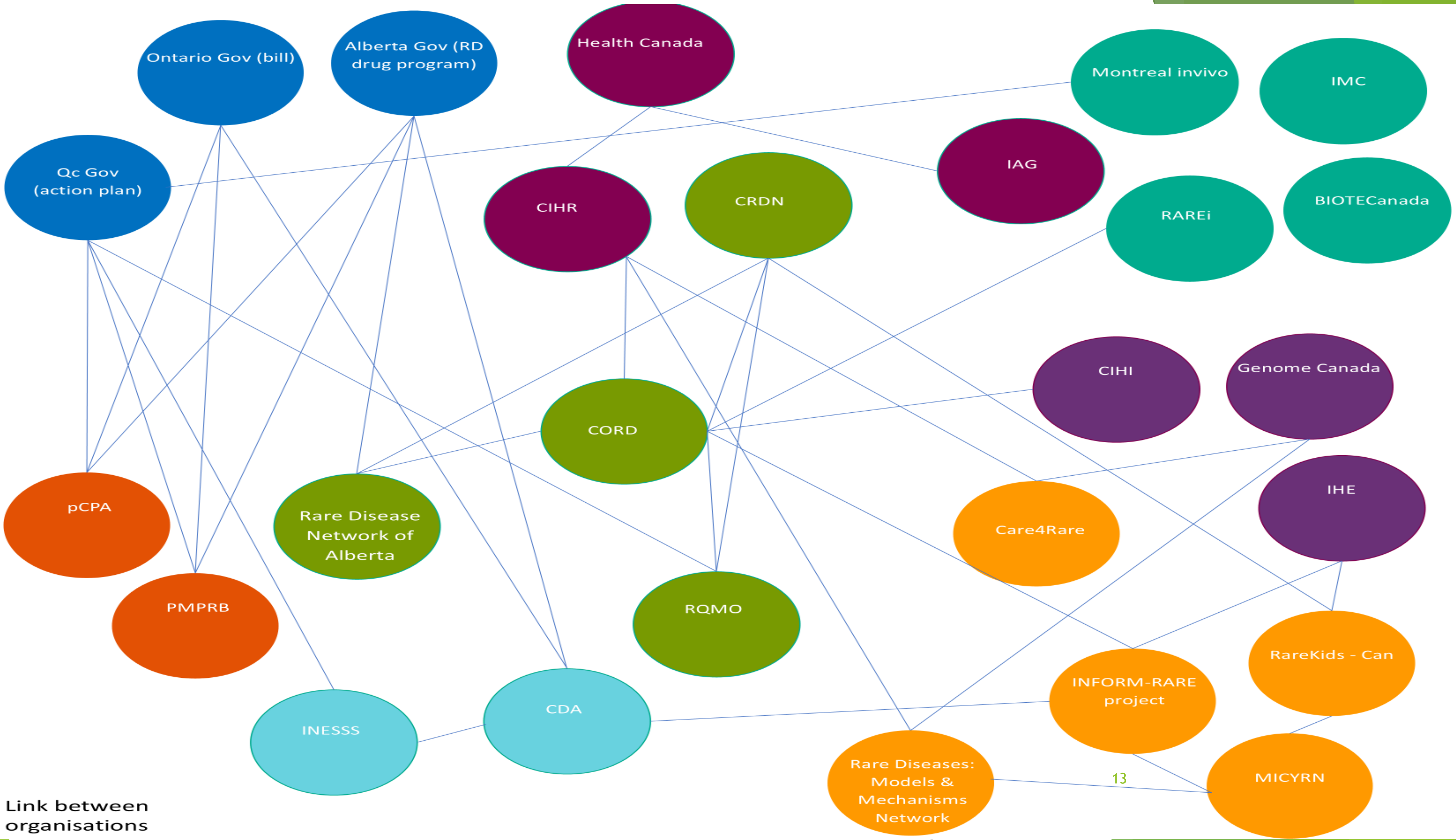
CRDN & RareKids

Policy Observatory

Industry associations

CIHI & CHRN

CADTH/INESSS



Link between organisations

What is the unmet need?

- A number of Canadian rare disease (RD) initiatives underway
  - BUT - there are no programs specifically targeting **RD policy research**, particularly as it relates to medication access and reimbursement policies

- Need for **new thinking, new processes, and combined effort** in policy research to move the Canadian RD ecosystem forward in an evidence-informed manner

EXAMPLE: SPANISH POLICY OBSERVATORY

SPEAKING OPPORTUNITIES

VIRTUAL MEETINGS WITH POLICY MAKERS AND CLINICIANS

THIRD PARTY EVENTS

- **Observatory Public Virtual Webinars**: Orphan Drugs P&R, Agility and equity Access to OD, Innovative contracting, Training and information for clinicians and RD patients, Early diagnosis solutions, RD Management evaluation, Orphan Drugs incentives, Social needs for RD patients
- **Andalusian Parliament** NBS Forum
- **1 OD World Congress**

EXTERNAL ENGAGEMENT

MEETINGS

WITH STAKEHOLDERS

Impact on decision makers, clinicians, scientific societies and other stakeholders.

Several **collaborating institutions.**

POLITICAL ACTIVITIES

POLICY INITIATIVES ON RD/OD IN LEGISLATURE

CONGRESO DE LOS DIPUTADOS

Grupo Parlamentario Popular

A LA MESA DEL CONGRESO DE LOS DIPUTADOS

El Grupo Parlamentario Popular en el Congreso, al amparo de lo dispuesto en el artículo 193 y siguientes del vigente Reglamento del Congreso de los Diputados, presenta la siguiente **Proposición no de Ley de medidas destinadas a mejorar la calidad de vida de las personas con enfermedades raras** para su debate en la **Comisión de Derechos Sociales y Políticas Integrales de Discapacidad.**

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Madrid, 24 de agosto de 2021

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Diego MOVELLÁN LOMBILLA
Carmen NAVARRO LACOBA
José ORTÍZ GALVÁN
Miguel Ángel PANIAGUA NÚÑEZ
DIPUTADOS

MEDIA CAMPAIGNS

Impact on **specialized and general public media**

RD & OD have captured the attention from **leading health media**.

Social media impacts

Focus for today….

- Introduction of the Canadian Rare Disease Policy Observatory (PO) concept
  - Serve as an evidence-support organization for rare disease policy
  - Provide a key tool for long-term policy research and stakeholder engagement

Policy Observatory – “an evidence-support organization for rare disease policy”

Proposal – A Canadian Rare Disease Policy Observatory

Dr. Rebeccah Marsh

Canadian Rare Disease Policy Observatory

VISION

Improved and equitable health outcomes for people impacted by rare diseases.

MISSION

Lead a coordinated effort to prioritize and address key policy issues that will enhance equitable and sustainable access to and reimbursement of innovative therapies for rare diseases.

Objectives of the Policy Observatory

1. **ALIGN** policy efforts in the rare disease space
2. **ADVANCE** regulatory and reimbursement policy solutions
3. **EVALUATE** the impact of policy initiatives and investments

Types of observatory activities

Policy Research & Analysis

Generating evidence-based insights to inform rare disease policies

Policy Strategy

Developing actionable strategies to implement effective and sustainable policies

Policy Engagement & Dialogue

Facilitating stakeholder discussions to identify, align on, and prioritize policy issues and solutions

Collective Learnings

Collecting and disseminating best practices and lessons learned

Principles guiding the work

EVIDENCE-BASED POLICY

Ground policy options in robust real-world evidence and research

SYSTEMS THINKING

Consider interconnections across sectors and how changes affect the broader system

GLOBAL PERSPECTIVE, LOCAL IMPACT

Learn from international best practices while tailoring solutions to Canada’s unique context

SOLUTIONS FOCUSED

Move beyond identifying problems to prioritize actionable and practical solutions

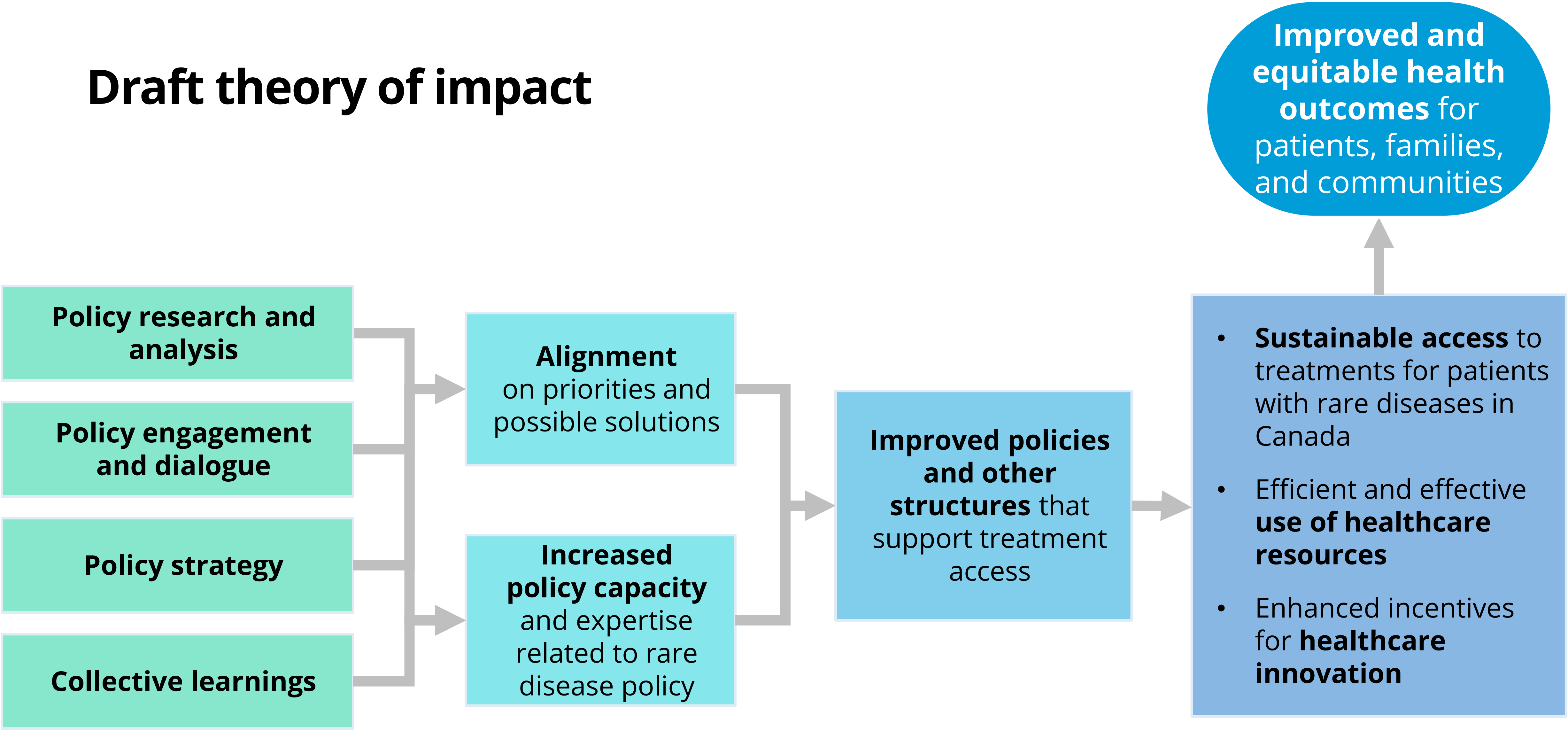
SUSTAINABILITY

Support policies that are effective, affordable, and sustainable long-term

EQUITY, DIVERSITY, INCLUSION

Focus on disparities in access and outcomes and ensure diverse representation and inclusivity

Draft theory of impact



Policy research and analysis

Policy engagement and dialogue

Policy strategy

Collective learnings

Alignment on priorities and possible solutions

Increased policy capacity and expertise related to rare disease policy

Improved policies and other structures that support treatment access

- **Sustainable access** to treatments for patients with rare diseases in Canada
- Efficient and effective **use of healthcare resources**
- Enhanced incentives for **healthcare innovation**

Improved and equitable health outcomes for patients, families, and communities

Cross-sector, multi-disciplinary initiative

Convened by neutral, independent non-profit organization

POLICYMAKERS

PATIENT GROUPS

HEALTHCARE PROVIDERS

IHE

MANUFACTURERS

RESEARCHERS

REGULATORS

A Patient Advocate Perspective

Dr. Gail Ouellette

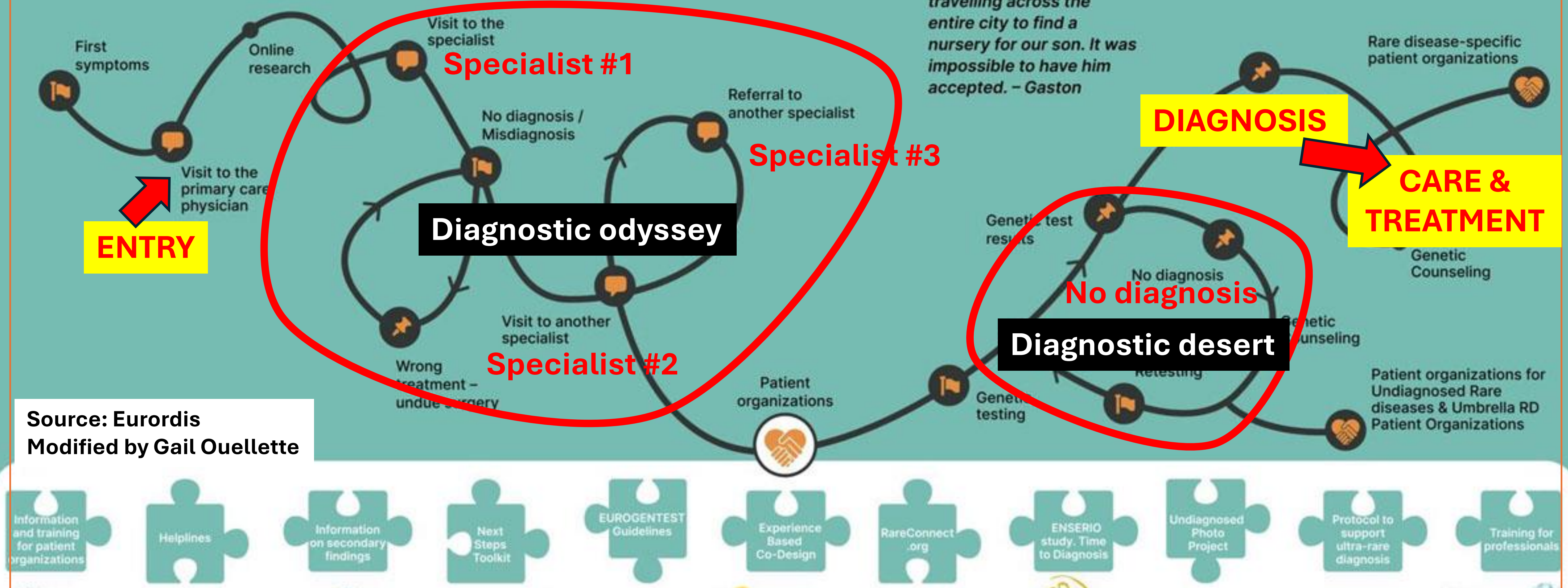
Patient Journey through diagnosis

> *It's a waiting game, but you tell a mum to wait when she's waited 15 years. It's difficult. – Nuria*

> *People began to ask which side of the family it came from...It was a difficult time for us as parents. – Alexa*

> *A diagnosis may be bad news, it may be very bad news or it may be no news. But all of that's OK and there's help and support for whatever spectrum you end up on. – Peter*

> *We went around, travelling across the entire city to find a nursery for our son. It was impossible to have him accepted. – Gaston*



First symptoms

Online research

Visit to the primary care physician

ENTRY

Visit to the specialist

Specialist #1

No diagnosis / Misdiagnosis

Diagnostic odyssey

Referral to another specialist

Specialist #3

Visit to another specialist

Specialist #2

Wrong treatment – undue surgery

Patient organizations

Genetic testing

Genetic test results

No diagnosis

No diagnosis

Diagnostic desert

Retesting

etic
unseling

DIAGNOSIS

CARE & TREATMENT

Genetic Counseling

Rare disease-specific patient organizations

Patient organizations for Undiagnosed Rare diseases & Umbrella RD Patient Organizations

Information and training for patient organizations

Helplines

Information on secondary findings

Next Steps Toolkit

EUROGENTEST Guidelines

Experience Based Co-Design

RareConnect.org

ENSERIO study. Time to Diagnosis

Undiagnosed Photo Project

Protocol to support ultra-rare diagnosis

Training for professionals

Source: Eurordis
Modified by Gail Ouellette

A Clinician Perspective

Dr. Hernan Gonorazky

A Policy-Maker Perspective

Marcel Saulnier

Audience Q&A

Wrap-up

Key Take-Aways For Today

- **CALL TO ACTION:**
  - Interested parties should engage with IHE and support the PO in an effort to align RD policy development efforts and advance regulatory and reimbursement policy solutions.
- **IMPACT:**
  - A Canadian Rare Disease Policy Observatory would help identify, prioritize, and fill-in the policy development gaps in support of informed decision making.

Housekeeping - Reminders

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  - Feedback on this session and the whole event would be greatly appreciated.

Thank You!

- Thank you to our panelists!
- Thank you to our audience!
- Thank you to CAPT for this opportunity!

Thank You!