

RARE

DISEASES

Centers of Excellence - Latin America

DURHANE WONG-RIEGER, PHD

NOVEMBER 2024

CONTENTS



General Context



Key Elements



Case Study: Colombia



Casa dos Raros: Brazil

BEHAVIOR OF CANADIAN SPENDING ON RARE DISEASE AND ITS PROJECTION

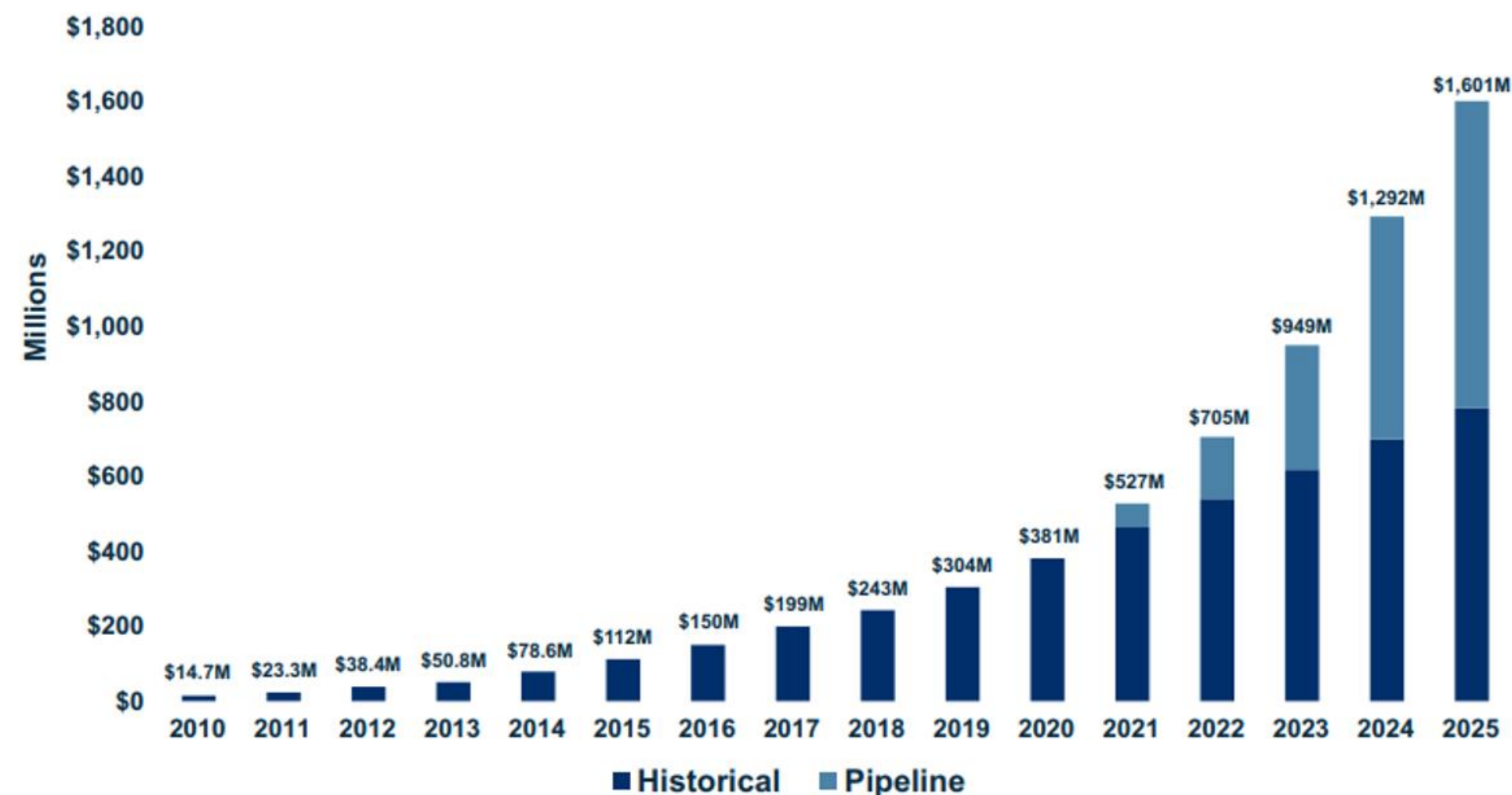


Fig. 2 Annual spending on historical and pipeline drugs for rare diseases between 2010 and 2025. Public spending on Health Canada-approved historical drugs for rare diseases (DRDs) was evaluated from 2010 (11 DRDs) to 2020 (42 DRDs). Linear extrapolation was used to project spending on historical DRDs during 2021–2025, and projected spending on pipeline DRDs was added to produce the total annual spending on historical and pipeline DRDs during 2021–2025. All amounts are shown in millions of Canadian dollars. Assumptions that guided our projections for pipeline DRDs are listed in Tables 1 and 2

Rare is frequent and frequent is costly: as a challenge for health care systems

J.-Matthias Graf von der Schulenburg ·
Martin Frank

Published online: 30 October 2014
© Springer-Verlag Berlin Heidelberg 2014

In many cases, the treatment of rare diseases is extremely costly. One example is the inherited disorder Gaucher's disease, which can be treated with an enzyme replacement therapy. A model-based cost-effectiveness analysis calculated lifetime costs of 5,716,473 euro for a patient with type 1 Gaucher's disease in a Dutch setting. However, the treatment is extremely effective. Over an 85-year lifetime period, 55.86 QALYs (quality-adjusted life years) were gathered, resulting in a cost-effectiveness ratio of 884,994 euro per QALY [10]. Comparable results were shown for enzyme replacement therapies for patients with Fabry disease [11]. A study analyzed the budget impact of orphan drugs in Sweden and France from 2013 to 2020, using a dynamic forecasting model. Orphan drugs caused 2.7 % in Sweden and 3.2 % in France of total drug expenditure in 2013. A portion of

**WE FOUND THAT THE TOTAL SPENDING
AND OUT-OF-POCKET HEALTH SPENDING
OF PATIENTS IN THE TOP 20% INCOME
GROUP WAS SIGNIFICANTLY HIGHER
THAN THAT OF THE BOTTOM 20%
INCOME GROUP..**

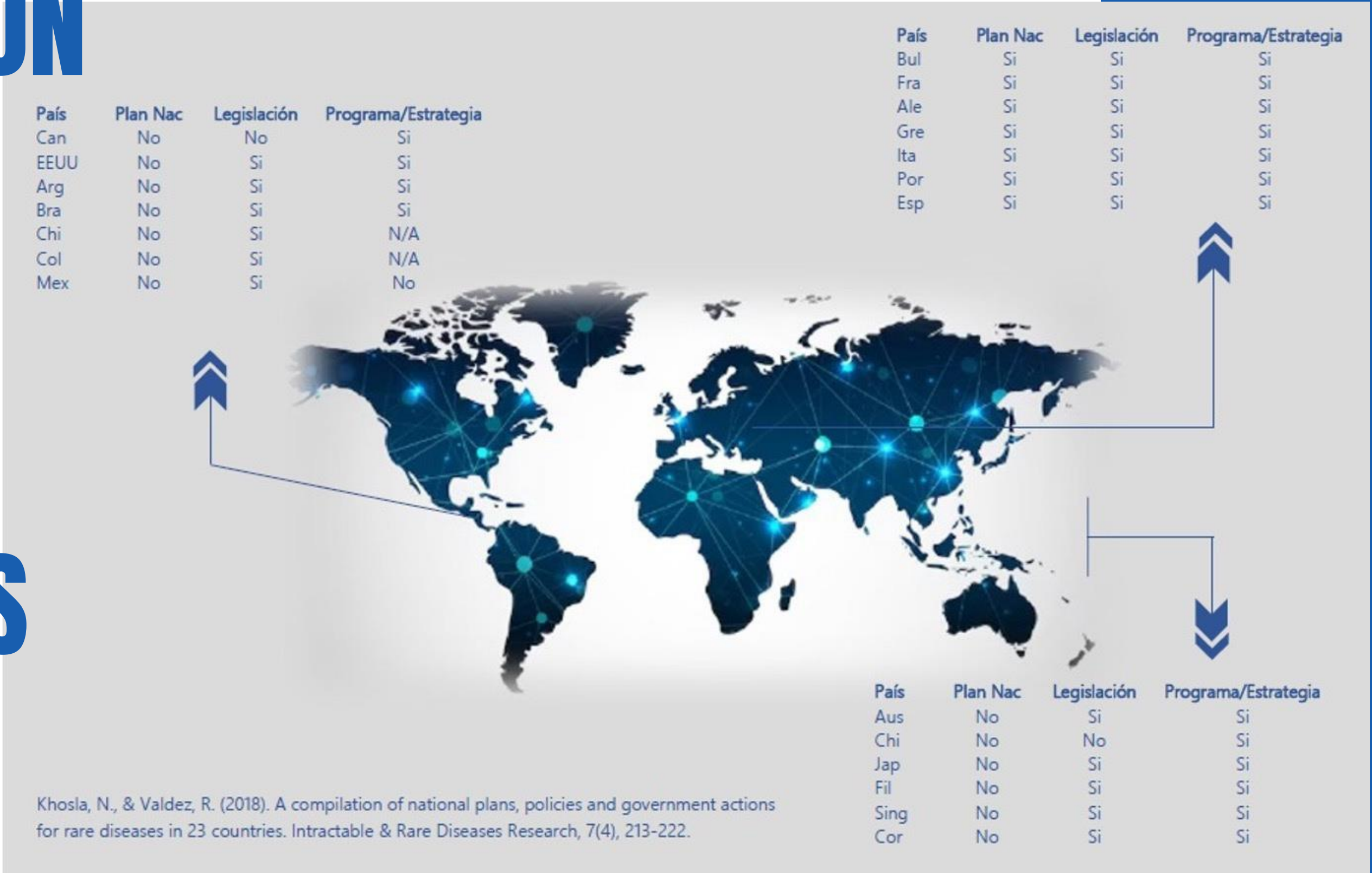
Xin, X. X., Zhao, L., Guan, X. D., & Shi, L. W. (2016). Determinants and equity evaluation for health expenditure among patients with rare diseases in China. Chinese medical journal, 129(12), 1387-1393.

KEY ELEMENTS



**PLANS AND REFERENCE
POLICIES**

A COMPILATION OF NATIONAL PLANS AND GOVERNMENT ACTIONS IN 23 COUNTRIES



REFERENCE PLANS

United Kingdom

France

India

Australia


Perú

Columbia

Panamá

European Union

COMISCA (Council of Ministers of Health of Central America and the Dominican Republic)



LONG TERM VISION

STATE ROADMAP

INSTITUTIONAL FRAMEWORK FOR COMPREHENSIVE POLICY DEVELOPMENT.

Intermediate and long-term objectives

Contingency response capacity

COMMON APPROACHES TO ACCESS

KNOWLEDGE MANAGEMENT

PATIENT PARTICIPATION

ESTABLISHED CAPACITY & PATHWAYS

FUNDING & SUSTAINABILITY

FUNDAMENTAL SOLUTIONS

Overcome the **false dichotomy** of dilemma
between **“us and them”**

Create conditions for progressive healthcare rights



United Nations

A/RES/76/132



General Assembly

Distr.: General
5 January 2022

Seventy-sixth session
Agenda item 28
Social development

**Resolution adopted by the General Assembly
on 16 December 2021**

[on the report of the Third Committee (A/76/454, para. 51)]

76/132. Addressing the challenges of persons living with a rare disease and their families

KNOWLEDGE MANAGEMENT

PREMISES

- Inform policymakers & patients
- Set incentives for data registration
- Digital ecosystem economy - interoperability

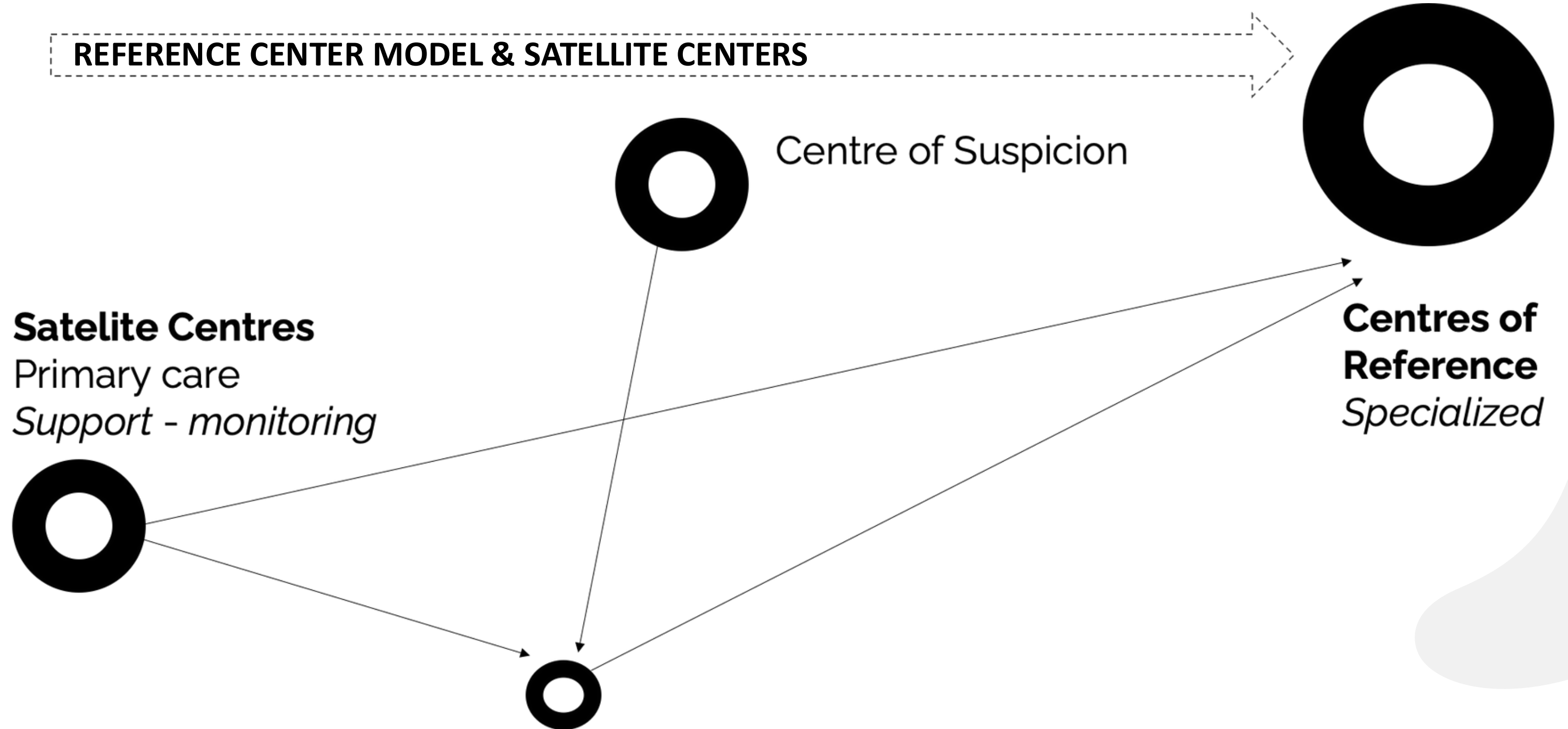
INSTITUTIONAL FRAMEWORK FOR DIALOGUE

Participation of Patients & Caregivers

PREMISES

- Social consensus requires compromise
- Build trust (continuity)
- Adopt a strategic framework and tactical approach
(achieving both large and small victories)

ESTABLISHED CAPACITY



RISK MANAGEMENT AND VALUE

**MODELS TARGETING THERAPEUTIC MILESTONES, QUALITY OF LIFE, AND
HEALTHCARE SPENDING EFFICIENCY**

Evidence generation, standards, and indicators

FUNDING

ECONOMIES OF SCALE

Mechanisms for centralized purchases with strong governance frameworks.

INNOVATIVE FUNDING APPROACHES

Managed access agreements, risk-sharing, social impact bonds, and multilateral bank leverage.

SOCIAL SPENDING ON SOCIAL PROTECTION

Increased health spending with a vision for social protection and efficiency: multisectoral coordination.

CASE STUDY

COLOMBIA

COLOMBIA

SUCCESSES & CHALLENGES

Sustainable funding
framework)

Long-term vision with
Law 1392 of 2010

Information system for
reporting orphan diseases

Centres of Reference

+ 90.000 patients

Incentives

Patient participation

CASA DOS RAROS

BRAZIL



Durhane Wong-Rieger, PhD

President & CEO

Canadian Organization for Rare Disorders



durhane@raredisorders.ca



Canadian Organization
for Rare Disorders

TAKEAWAYS

BUILD A LONG-TERM VISION
THROUGH CONSENSUS—
CONSTRUCTIVE DIALOGUE

INFORMATION MANAGEMENT WITH
INCENTIVES

INSTALLED CAPACITY IS IMPORTANT,
BUT A **VALUE-BASED MODEL**, IS EVEN
MORE CRUCIAL