



RDI Perspective: Access to treatment in LMCs

**IPWSO Summit
November 12th, 2024**



**RARE
DISEASES
INTERNATIONAL**



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RARE
DISEASES
INTERNATIONAL

2025



About Rare Diseases International

The **Global Alliance** of Persons Living with a Rare Disease and their families
across **all countries** and **all rare diseases**

A GLOBAL MEMBERSHIP BASE

Active in over

150 COUNTRIES

covering **6** continents



116

MEMBER organizations

RD NATIONAL ALLIANCES
Represent all RDs in one country

49

4

REGIONAL ALLIANCES

INTERNATIONAL FEDERATIONS
Represent the same RD or disease area internationally

56

7

MULTI-STAKEHOLDER
PLATFORMS



OUR VISION

A world where PLWRD and their families experience a **BETTER LIFE** through full recognition and support

OUR MISSION

A **STRONG COMMON VOICE** for PLWRD and their families around the world

OUR STRATEGIC GOALS

1

Build capacity and enhance member engagement to increase influence and reach

2

Influence the global policy ecosystem for prioritization of rare diseases and sustainable resources for rare diseases

3

Improve access to treatment and care for PLWRD through care pathways and networks for rare diseases

4

Drive better outcomes for PLWRD through strategic partnerships

IMPROVE EQUITY FOR PLWRD

OUR IMPACT STRATEGY

Our Vision	A world where Persons Living with a Rare Disease (PLWRD) and their families experience a better life through full recognition and support.							
Our Mission	Be a strong common voices for PLWRD and their families around the world							
Our Values	Global	Equitable	Inclusive	Diverse	Person-centered	Independent	Collaborative	Innovative
Our Strategic Goals	Improve EQUITY for PLWRD around the world							
	Community Engagement Enhance capacity and engagement of rare diseases community in the response	Prioritization of rare disease in policy Financing for adequate and sustainable resources for rare disease care	Care pathways and networks Improve access to treatment and care for PLWRD					
Our Strategic Pathways								
	STRATEGIC ENGAGEMENT Building capacity fellowships, toolkits and resources, trainings, grants Strengthening regional alliances regional events, dialogues and collaboration Raising awareness Rare Disease Day, communication and awareness raising	GLOBAL ADVOCACY Shaping the global policy ecosystem UN Resolution on PLWRD and their families UN Political Declaration on UHC Advocating for PLWRD as a global advocacy priority WHA Resolution, Global Action Plan Partnering on a global and regional scale UHC2030, NGO Committee for Rare Diseases	GLOBAL PROGRAMMES Improving recognition of rare diseases Operational Description of RD Supporting timely diagnosis Newborn screening, WHO EDL, Global Commission Strengthening health systems Global Network for Rare Diseases (GNRD) Advancing equitable access Access Working Group, GardAccess, WHO EML, IRDIRC	STRATEGIC PARTNERSHIPS Fostering partnerships for impact Foundations, Philanthropic organizations, public sector donors Engaging the private sector RDI Alliance of Companies Sustainable resource development				
Our Strategic Enablers	Governance and Membership	Team and organizational culture	Operations and Infrastructure	Financial Sustainability and Efficiency				

Identification of Access barriers

2022-2023 Global Access Working Group

20 members from 14 countries:

- 8 **RDI** member organisations
- Regulator
- Clinician
- Pharmacist
- Researcher
- Industry
- **IRDiRC**



Objective

Research into **barriers to accessing RD medicines**

Approach

Gather real experiences and **cases studies** regarding access to medicines, and establish a framework for access to promote changes in a systemic way.

Case Study I: CYSTINOSIS

Interviews carried out in **11** countries: Australia, Canada, Colombia, Ghana, Malaysia, Mexico, New Zealand, South Africa, Spain, Thailand, Vietnam

Case Study II: CYSTIC FIBROSIS

Interviews carried out in **7** countries: Brazil, Ireland, Israel, Italy, Latvia, South Africa, Turkey

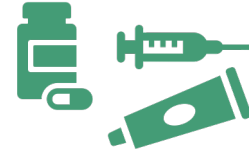
Lessons learnt

A wide range of access challenges reported in different countries settings.

Availability of diagnosis and early diagnosis are essential to being timely treated and care planning for all medical conditions.

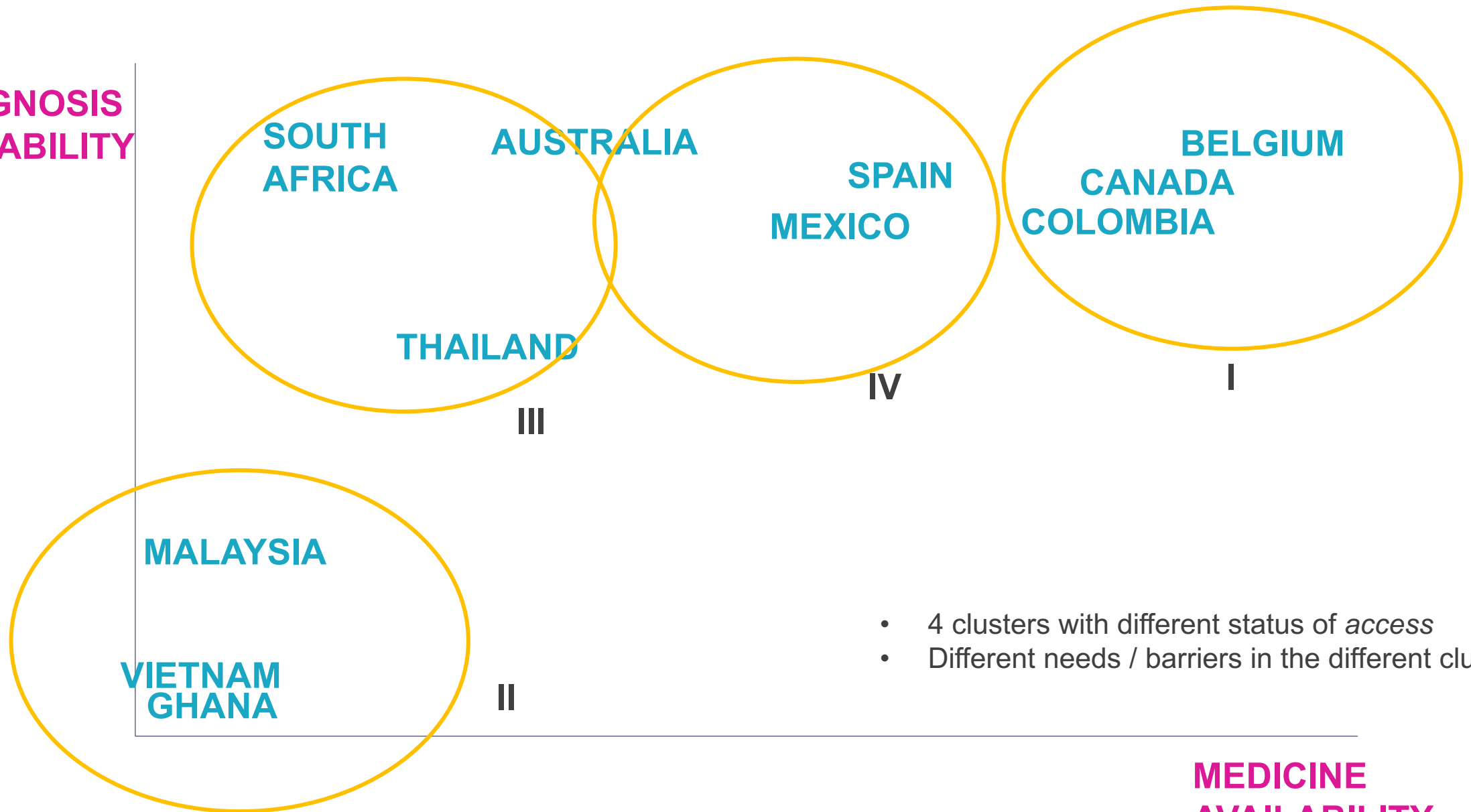
What are the pain points?

Each pain point may be felt by each stakeholder in a different way



No access to life saving medications	Able to only provide symptom	Roll out of global marketing takes a long time	
Out-of-Pocket payments, causing financial burden and difficult family choices (relocation, children)	Heavy administrative burden to prepare repeated applications for import and approval.		Is there a policy in place to expedite OD approval / path for import?
Delays in HTA after medicines approval		Negotiation with government agencies may take a long time.	Law suits enforced reimbursement which does not have the best value
Compounded medicines which have different quality, affecting efficacy and compliance			Oversight on quality
Taste and dosage, which can influence compliance in children			
Lack of access to trials			Unable to attract investment
Delayed/long diagnosis	Lack of confirmatory tests, too expensive to send samples abroad		


**DIAGNOSIS
CAPABILITY**



- 4 clusters with different status of *access*
- Different needs / barriers in the different clusters

**MEDICINE
AVAILABILITY**

Common Access Challenges



Lack of validation tests
Lack of experienced labs
Lack of awareness
Limited workforce

Out of pocket payments
Import rules & procedures
Negotiations with distributors
Compliance issues
Logistical risks

Quality of the products
Formulation strengths
Timely reimbursement
Knowledge sharing
Care arrangement
Transition from pediatric to adult care
Support for carers

Elements to improve access



Existence of
patient support
groups



Supportive
clinicians



Clear regulatory
pathway



Clear importation
pathway



Efficient
regulatory to HTA
process



Exchanges
among experts

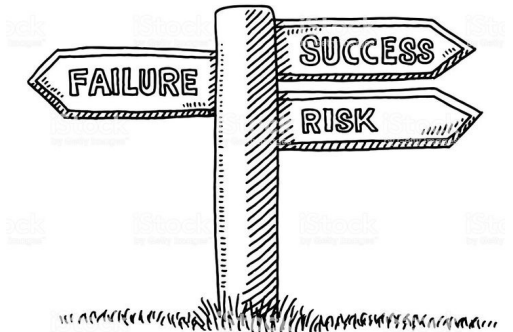
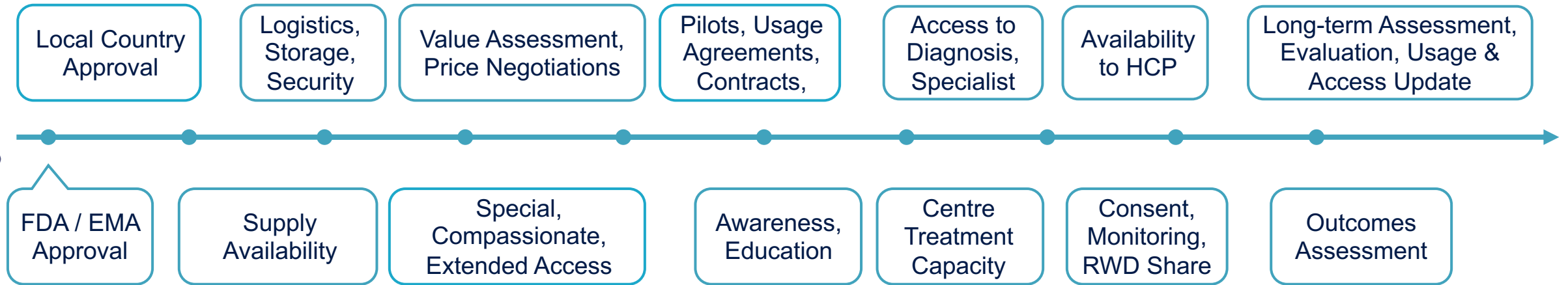


Alternative
diagnostic routes

The 4As of access to medicines



Framework for Access



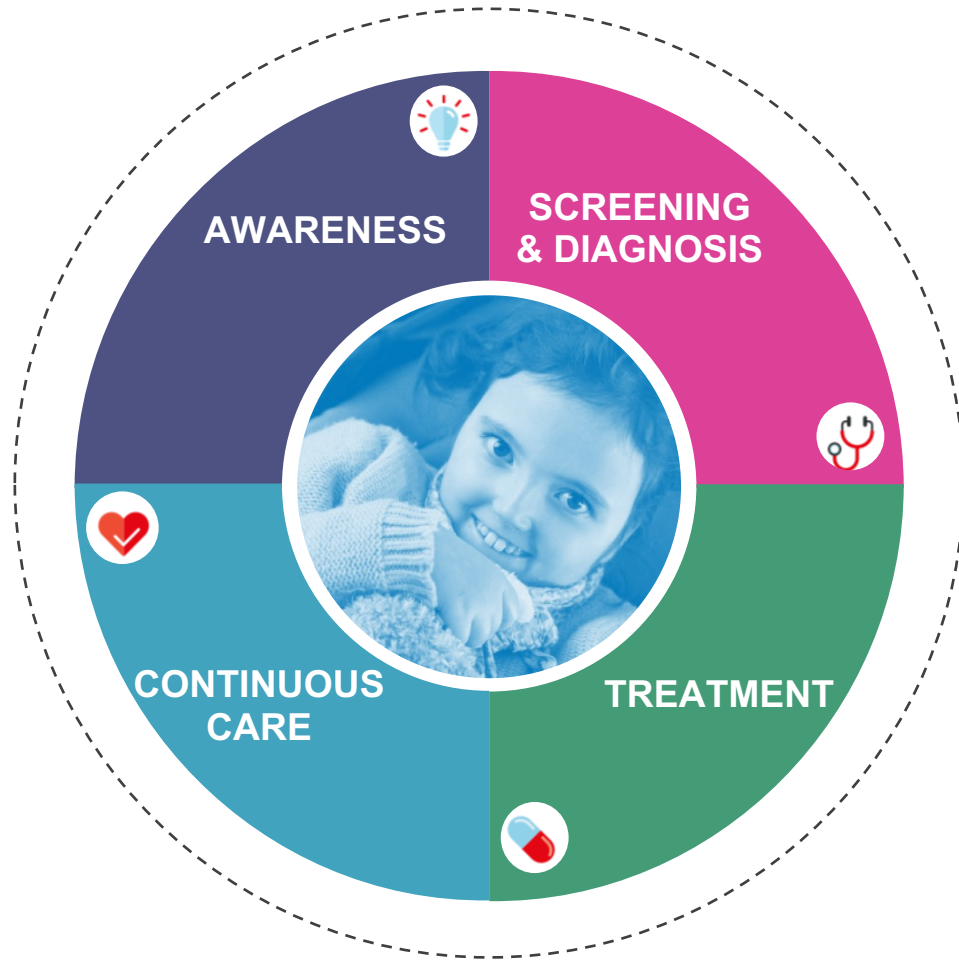
Roadmap to navigate the maze?



Needs – based Roadmap with actions to improve access

- 4 – 5 clusters of needs with different status of access
- Which are the low hanging fruits?

Making an impact



To ensure inclusive patient access to the support and resources needed for optimal health.

WHA Resolution Rare Diseases and GAPRD

WHA Resolution 2025

asks the WHO Director-
General (DG) to develop a
Global Action Plan on Rare
Diseases (GAPRD)



Global Action
Plan on Rare
Diseases
2027

Aims of the Resolution

Objective 1

To promote and enhance universal health coverage (UHC) to improve public health and social measures needed to support PLWRD, their families and caregivers.

Objective 2

To strengthen the efforts of Member States and all stakeholders in order to raise awareness, improve diagnostic capabilities and foster research and development of therapies, ensuring that PLWRD have access to affordable, effective, and comprehensive healthcare services, regardless of the rarity of their condition, without facing financial hardship.

In order to achieve the main objectives of the Resolution, the primary proposed aim is **to call upon the WHO to develop, in consultation with Member States, and in collaboration with relevant non-State actors, a comprehensive Global Action Plan on Rare Diseases.**

WORLD HEALTH ASSEMBLY (WHA) RESOLUTION ON RARE DISEASES – CURRENT STATUS



























Egypt initiating: *“Resolution on Rare Diseases: A Priority for Global Health Equity and Inclusion”*

Countries officially **co-sponsoring**: **Qatar, Spain, Malaysia, France, Panama, Chile**

Status: cosponsors are drafting the resolution text; Tabled for the WHO Executive Board 3 to 11 February 2025

Main Ask: Development of **Global Action Plan on Rare Diseases** (general alignment by co-sponsors)

Engagement ongoing to recruit more Member States as co-sponsors

Commitment secured		Engagement Underway		Important but not yet engaged	
Egypt		Ghana		Ecuador	
Qatar		Singapore		Kenya	
Spain		Argentina		New Zealand	
France		Brazil		USA	
Malaysia		Australia		Thailand	
Panama		Switzerland		Senegal	
Chile		UK		Zimbabwe	
		Canada		Barbados (EB Chair)	
		Lesotho		Norway	
		Japan			
		China	