



Rare Diseases International

CNA and CEF Joint Meeting
27 November 2020

Flaminia Macchia, Hlawulani Mkhabela and Clara Hervas



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DISEASES
INTERNATIONAL

Who are we?

Rare Diseases International (RDI) is the global alliance of people living with a rare disease of all nationalities, across all rare diseases



A Global Rare Disease Community



Our Mission

To be a **strong common voice** for people living with a rare disease (PLWRD) around the world



Our Vision

To ensure that people living with a rare disease and their families **experience better recognition and support**, improved health and social services, and overall a better life



Our Objectives

- To **advocate for rare diseases as an international policy priority** (research, public health, human rights) through awareness raising, policy promotion and international collaborations
- To **represent** people living with a rare disease and their families **at international institutions and fora**
- To **enhance the capacities of our members** through information exchange, networking, mutual support and joint actions



RDI Members

Our members are **patient-driven rare disease organisations** spanning the globe. Active at national, regional and international level.



RDI Members – Nov. 2020

Active in over
100 COUNTRIES



74

Member Organisations

22

International Federations

Represent the same RD or disease area internally or regionally

41

RD National Alliances

Represent all RDs and one country



3

RD Regional Alliances

Represent all RDs in a geographical region

Europe (EURORDIS), Ibero-America (ALIBER) and Asia Pacific (APARDO)

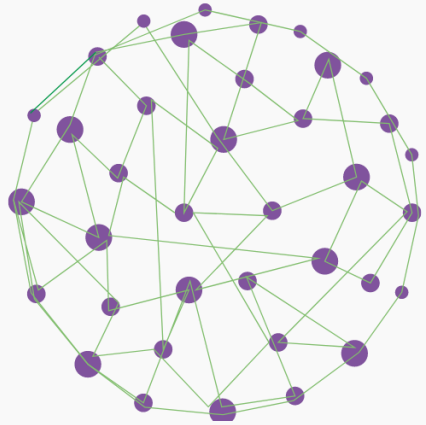


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Multi-stakeholder Platforms and Other RD Organisations

Associate Members

Connecting and Engaging Members



- **Regular consultation** to shape and participate in RDI's advocacy, programmes and activities
- **RDI Annual Meeting** – Membership Meeting and the RDI Global Meeting, an event that brings together RD stakeholders across the globe
- Opportunity to participate in various online and in-person **rare disease conferences and events** throughout the year
- **RDI support for your rare disease events** (communication, speakers, fellowships)

● **Monthly RDI newsletter** – to share news, events, calls for input and action

● **Online communities** for RDI members to connect, share news and best practice

● National Alliances are **official partners of the global Rare Disease Day campaign**



RARE DISEASE DAY
RARE DISEASE DAY.ORG



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Governance: RDI Council



**Durhane
Wong-Rieger**

**CORD
Canada**



**Lisa
Sarfaty**

NORD



**Yann
Le Cam**

EURORDIS



**Ritu
Jain**

**DEBRA
International**



**Kin Ping
Tsang**

**Rare Disease
Hong Kong**



**Rachel
Yang**

**CORD
China**



**Alba
Ancochea**

**FEDER
Spain**



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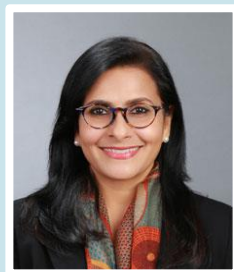
Governance: RDI Advocacy Committee



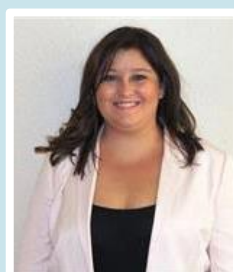
Yan Lee Cam
EURORDIS
Chair



Maureen Smith
CORD-Canada



Ritu Jain
DEBRA
International
APARDO



Kelly du Plessis
RDSA-South
Africa



Ramaiah Muthyala
IORD-India



Lara Bloom
Ehlers-Danlos Society



Leire Solis
IPOPI



Luciana Escati
FADEPOF-Argentina



Eleni Antoniou
Thalassaemia
International



Sanja Peric
RD-Croatia



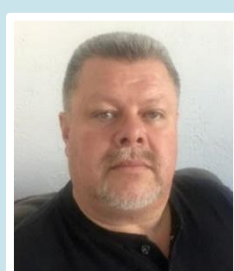
Migdalia Denis
Latin PH Society



Lieven Bauwens
Child-Help



Alba Ancochea
FEDER-Spain



Jesús Navarro
OMER-Mexico
ALIBER



Simone Boselli
EURORDIS



Kawaldip Sehmi
IAPO



**Roberta Anido
de Pena**
FADEPOF-Argentina



Global Advocacy

- **Rarity calls for advocacy at the global level**
- **Leveraging the credibility and impact gained** by the rare disease community **in certain regions** to inspire and support RD advocacy around the globe
- Countries increasingly **align domestic policy with international commitments** (SDGs 2030, Universal Health Coverage), potentially benefiting PLWRD



UN Advocacy Areas



UN Advocacy Pathways

New York



Geneva



How?



Advocate UN Member States' Permanent Missions (New York and Geneva), HRC/OHCHR and Special Rapporteurs

Engage operationally with the Secretariat of the WHO

Organise and attend UN policy events

Consult and harness RDI's membership to engage national reps and other stakeholders



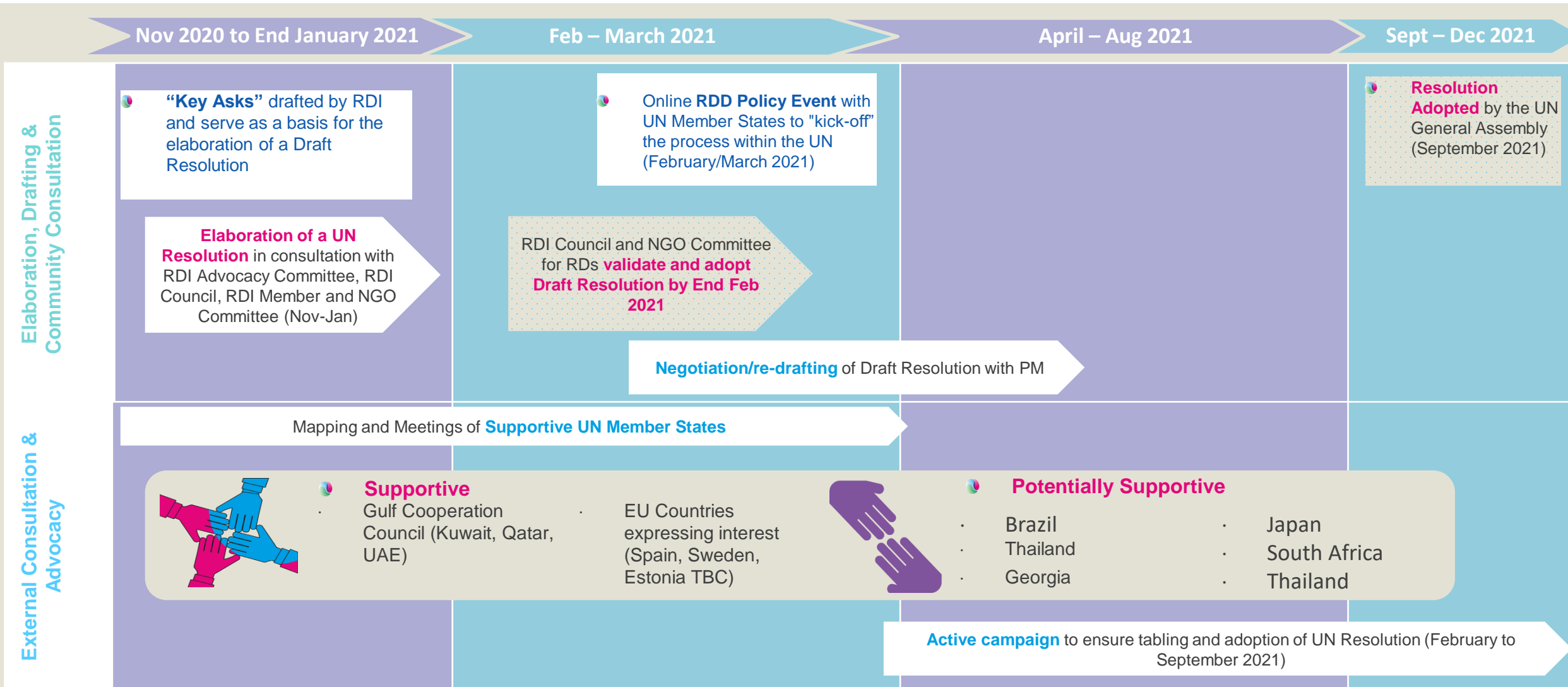
“UN Resolution to Address the Challenges of People Living with a Rare Disease”

Adoption of a UN Resolution to address the challenges of People Living With a Rare Disease:

- **Highest recognition of RDs** in the UN System
- **Advance RDs as a policy priority** for UN Agencies and Programmes
- **Promote the adoption of national strategies** for RDs in the 193 UN Member States



Timeline: UN Resolution on RDs



#UHC4RareDiseases

Implementing UHC for Rare Diseases

- Campaign **launched in October 2020 by EURORDIS and RDI** to advance the implementation of a commitment to RDs included in the UN Political Declaration on UHC
- The **#UHC4RareDiseases Toolkit** offers modifiable and translatable advocacy and communication materials:
 - **Template letter for national policy makers**
 - **Factsheet & Policy recommendations**
 - **Briefing Document**
 - **Social Media Pack**
- To mark **UHC Day 2020, RDI will host a webinar** co-organised with EURORDIS – an open dialogue on how UHC can support national and regional rare disease policy and programmes



Addressing Rare Diseases through UHC
UHC Day Webinar



Global Collaborations

- **Rarity calls for action and collaboration at the global level**
- **We join forces across stakeholders, from the WHO to global multi-stakeholder consortiums**
- **Through collaborative engagement, RDI is helping leverage unprecedented scientific and technological opportunities to advance rare disease diagnosis, care and treatment**



Working with the World Health Organization

- **RDI and WHO signed a Memorandum of Understanding**, December 2019
- Collaboration based on the WHO's 13th General Programme of Work, 2019-2023
- The Understanding aims to address public health needs and issues related to rare diseases, and help ensure that **UHC is supported by "healthcare systems strengthening through global networking of specialized services"**.

2020-2021 deliverable:

- **Operational description of RDs** and prevalence/incidence figures
- **Need assessment study** as basis to develop a methodological framework to identify, accredit, support and connect expert centres
- To develop a **Conceptual Model for a Global Network of multi-disciplinary expert centres for Rare Diseases (CGN4RD)**

- MoU will support RDI becoming a Non-State Actor in Official Relations with WHO



On 18 March 2019, Yann Le Cam and Durhane Wong-Rieger met with Dr Tedros, WHO Director-General, in Geneva

WHO Collaborative Global Network for Rare Diseases (CGN4RD)

- Vision: **Rare Disease Centres of Expertise connected internationally into one global network**
- Structure: **Global RD Hubs** of reference serving countries and regions and building local capacity and expertise
- Multi-stakeholder Engagement: Patient organisations, health system providers, specialists, family physicians, policy makers

• **Timeline:**

- 2020 – 2024 Discovery, Development and Pilot
- 2025 – 2029 Scaling up and Operational Network
- 2030 – 2034 Evaluation and Renewal

Programme Activities (2020)

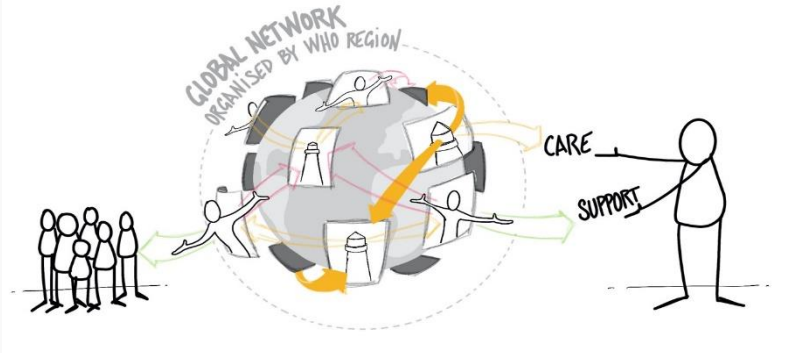
- Needs assessment
- Literature review
- Semi-structured Interviews

WHO Deliverables:

- Needs Assessment Study
- Concept & Methodological Model

Member Engagement Plan (2020 -2021)

- **RDI Members through focus groups and call for input** to identify needs and healthcare landscape: 7 Regional Focus Group & International Federations Meeting
- **Support to Regional / National Alliances** to engage their Members



Member of the International Rare Disease Research Consortium

An international multi-stakeholder collaboration to **advance rare diseases research worldwide:**

- RDI is a member of the **Patient Advocacy Constituent Committee** – 13 of 15 patient reps in the Committee are RDI Members organisations
- Treatments are often unavailable for rare disease patients, especially in low and middle income countries. RDI Co-chairs the **Access to Medicines Working Group** to help facilitate access to treatment in LMICs.

Access Working Groups Objectives:

- Create a **list of standard-of-care products for rare diseases** to be made available to all countries and periodically updated
- **Identify the barriers to accessing rare disease treatments**, particularly in low-and-middle income populations



IRDIRC

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RARE DISEASES RESEARCH
CONSORTIUM



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Member of the Global Commission

The GC was established by Takeda, Microsoft and EURORDIS to **improve diagnostic pathways for children with a rare disease:**

- **Four RDI Members are GC Members:** Yann Le Cam (EURORDIS), Durhane Wong-Rieger (CORD Canada), Kevin Huang (CORD China), Pamela Gavin (NORD)
- RDI is active in the two working groups:
 - **Empowerment & Awareness Working Group** - Broaden awareness about the need to reduce time to diagnosis and empower key stakeholders to accelerate diagnosis
 - **Policy & Advocacy Working Group** - Advocate for local and global policy change to build an enabling environment for rare disease diagnosis
- As the GC's activities move beyond the pilot phase, **RDI ensures that patient organisations from across the globe can play a role in the GC activities**



GLOBAL COMMISSION

to End the Diagnostic Odyssey for Children with a Rare Disease



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COVID-19

An **unprecedented global crisis**, threatening to exacerbate the vulnerability of people living with a rare disease, has stimulated **an unprecedented response** from the rare disease community.

RDI Members **continue to support PLWRD** through the pandemic and advocate to strengthen health and social systems to address rare diseases and **“build back better”**



COVID-19 Response

- **Develop RDI COVID-19 Resource Center** to provide essential information resources and surveys developed by the rare disease community
- **Host online meetings and consultations with members** to understand the impact on PLWRD and patient orgs around the globe
- **Survey** RDI Members to help map different national contexts as well as the concerns and needs of patient orgs. The survey forms the basis for RDI's Official Statement on COVID-19 Response and Recovery, July 2020

“
The health, social and economic impact of the pandemic should not further exacerbate the vulnerability of over 300 million people living with a rare disease.
”



Durhane Wong-Rieger
RDI Chair

RDI published a common statement with its international membership to call for PLWRD to be considered a **high-priority population in COVID-19 response and recovery plans:**

- Warns of the risk of exacerbating pre-existing inequalities
- Calls for the protection of PLWRD in COVID-19 response and recovery strategies and health system restructuring in the context UHC to ensure PLWRD are not left behind

- **Engage and inform WHO and the UHC2030** civil society alliance on the experiences and needs of PLWRD
- **Interact with international civil society partners** including the Non-Communicable Disease Alliance

Contact RDI



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Become a Member : <https://www.rarediseasesinternational.org/become-a-member/>



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Questions?