MEMBERSHIP MEETING
26 May 2021
14.00-17.00 CET

"Sibling Love" - Ataxia Telangiectasia - South Africa
UN RESOLUTION ON ADDRESSING THE CHALLENGES OF PERSONS LIVING WITH A RARE DISEASE AND THEIR FAMILIES
(45 min)
STATE OF PLAY & TOOLS
CLARA HERVAS, RDI PUBLIC AFFAIRS MANAGER
WHAT TO DO AND WHAT HAS BEEN DONE
PANEL DISCUSSION AND Q&A
YANN LE CAM, RDI ADVOCACY COMMITTEE CHAIR & RDI COUNCIL MEMBER
ROBERTA ANIDO DE PENA, FADEPOF FEDERACION ARGENTINA DE ENFERMEDADES POCO FRECUENTES (FADEPOF)
SAMUEL WIAFE, RARE DISEASE GHANA INITIATIVE
ELIZABETH VROOM, WORLD DUCHENNE

GLOBAL STATE OF THE ART RESOURCE (15 MIN)
INTRODUCTION TO GLOBALSOA.
FEEDBACK FROM MEMBERS’ CONSULTATION, WEBINAR AND SURVEY
FLAMINIA MACCHIA, RDI EXECUTIVE DIRECTOR
CHARLOTTE RODWELL, PARTNERSHIPS, BUSINESS DEVELOPMENT & STRATEGIC COMMUNICATIONS, ORPHANET & INSERM

WHO COLLABORATIVE GLOBAL NETWORK FOR RARE DISEASES: KICK OFF THE SECOND CYCLE (30 MIN)
PRESENTATION OF THE CONCEPT: ARE WE GOING IN THE RIGHT DIRECTION?
MATT BOLZ-JOHNSON, RDI PROGRAMME DIRECTOR, CGN4RD
PANEL DISCUSSION: Q & A
MEOW KEONG THONG, MALAYSIAN RARE DISORDERS SOCIETY
MATT BOLZ-JOHNSON & FLAMINIA MACCHIA

PAPER ON ESSENTIAL MEDICINES LIST, THE WHO ESSENTIAL IN VITRO DIAGNOSTICS LIST AND IRDRC ACCESS TASK FORCE (20 MIN)
DURHANE WONG-RIEGER, RDI COUNCIL CHAIR

CLOSING MESSAGE (5 MIN)
LISA SARFATY, NATIONAL ORGANIZATION FOR RARE DISORDERS (NORD)

SOCIAL NETWORKING (1 HOUR)
LET’S GET TOGETHER (CLICK ON LINK IN ZOOM CHAT)
UN RESOLUTION ON ADDRESSING THE CHALLENGES OF PERSONS LIVING WITH A RARE DISEASE AND THEIR FAMILIES

STATE OF PLAY & TOOLS
CLARA HERVAS, RDI PUBLIC AFFAIRS MANAGER

RDI Membership Meeting
26 May 2021
In this presentation

1. State of play: why a UN Resolution and how to get there?
2. Resolution4Rare tools
3. Launch of the ‘Dear UN’ campaign: a call for testimonies
State of play: Call for a Resolution4Rare
Civil society partners

Common vision: Advocating to elevate the issue of rare diseases as a global public policy priority within the United Nations

Global alliance of organisations of persons living with a rare disease

Legitimate platform to interact with the UN System

European alliance of organisations of persons living with a rare disease holding special consultative status with ECOSOC (UN)
Why a UNGA Resolution on RDs?

• A step ahead towards global recognition and visibility
• Set in motion a wave of policy actions – a catalyst
• Become a point of reference to substantiate the claims of the rare disease community at national level
• Help the development of national strategies, as well as the creation of international collaboration.
• Contribute to the achievement of the SDGs and the UN ambition to “leave no one behind”.
A contribution towards the Sustainable Development Goals

- The motto ‘to leave no one behind’ at the core of the UN Agenda 2030 Sustainable Development Goals, is fit for purpose for persons living with a rare disease

- The challenges of persons living with a rare disease have to be addressed in order to reach a significant number of these SDGs:
Key Asks of the Resolution4Rare campaign

1. HUMAN RIGHTS & INCLUSION

Participation of persons living with a rare disease & their families in society and respect of their human rights
Support the improvement of health and social outcomes with the appropriate care, and within existing resources.
Key Asks of the Resolution4Rare campaign

Promotion of national strategies and actions to leave no one behind
Key Asks of the Resolution4Rare campaign

4

INCLUSION IN THE UN SYSTEM

Introduction and recognition of rare diseases into UN agencies and programmes
Key Asks of the Resolution4Rare campaign

Regular reports by the UN Secretariat to monitor the progress on the implementation, including feedback from civil society.
Official launch at RDD Policy Event – 4th March 2021

- Patient testimonies from NZ, Malta, Kenya, Ghana
- Champion’s message: Nata Menabde, Director of WHO Office in NYC
- Support statements from 6 Ambassadors to the UN: Brazil, Qatar, Spain, France, Japan and Thailand
- Address from Todd Howland, OHCHR
Advocating at the UN: Why the General Assembly?

- The General Assembly assembles 193 Member States.

- Each Member State of the UN has one vote in the GA, but they like to form groups of like-minded partners (EU, small island states...)

- They focus on key topics for us:
  - Agenda 2030 & the Sustainable Development Goals
  - The goal to « Leave no one behind »
  - Universal Health Coverage
  - Sustainable Development: economic, social & environmental dimensions
Advocating at the UN: Why the General Assembly?

• The UNGA organises its work in Six Main Committees:

1st COMMITTEE
Disarmament & International

2nd COMMITTEE
Economic & financial

3rd COMMITTEE
Social, humanitarian & cultural

4th COMMITTEE
Special political & decolonization

5th COMMITTEE
Administrative & budgetary

6th COMMITTEE
Legal

• The Resolution4Rare will be presented through the 3rd Committee on Social, humanitarian and cultural issues
What is a UNGA Resolution?

- A UNGA Resolution represents the view of the 193 Member States
- Reflect the degree of intergovernmental agreement, the evolution of political ideas and the state of global cooperation on a given topic.
- It is not binding on MS but acts as soft law, and recalls important conventions and treaties that are binding
- It is binding on the Secretariat (DESA, OHCHR) and UN Funds & Programmes (UNDP, UNICEF)
- It is not binding on Specialised Agencies (WHO, World Bank, IMF) – but influences them as the de facto coordinator of UN system
The role of Permanent Missions to the UN

- All Member States have a UN Permanent Mission to the UN in New York.
- Diplomats from the Permanent mission seating on the 3rd Committee will meet in October and November to discuss resolutions and recommend them to the UNGA Plenary.
- The Plenary meets, usually in mid-December, to consider and adopt recommendations and then they become UNGA resolutions.
- During the process, diplomats ask for approval to their capital (Ministry of Foreign Affairs). Often, different ministries will be involved in the decision: Ministry of Health or Ministry of Social Affairs.
- Getting the support of the Ministries at national level is therefore key in the process!
Core group of Member States

- Working meetings in March & May 2021
- Will develop ‘zero draft’ of UNGA Resolution
- Will table it under agenda item of the 3rd Committee of the UNGA
- Will lead negotiations on the draft with other UN Member States
- Will co-organise side-events to gather support from other UN Member States
Other supportive UN Member States

France
Estonia
Thailand
Kuwait
Japan
Italy
The Resolution4Rare tools
The Resolution4Rare Toolkit

Toolkit featuring regular and new uploads every month including:

• Letter templates to send to policy makers: Ministry of Health, Social Affairs or Foreign Affairs/Permanent Missions to the UN

• Concept Notes to help you talk about it

• Factsheet

• Posts, images and GIFs to spread the word on social media using the hashtag #Resolution4Rare
The Resolution4Rare Toolkit

Facebook, LinkedIn & Twitter

Key Asks – What is the rare disease community asking for:

HUMAN RIGHTS & INCLUSION

APPROPRIATE CARE

INCLUSION IN THE UN SYSTEM

NATIONAL ACTION

MONITOR PROGRESS

Infographics & Factsheets

ADDRESSING THE CHALLENGES OF PERSONS LIVING WITH A RARE DISEASE AND THEIR FAMILIES

KEY ASKS

TO ADDRESS THE CHALLENGES OF PERSONS LIVING WITH A RARE DISEASE AND THEIR FAMILIES

1. HUMAN RIGHTS & INCLUSION
   - Participation in decision-making processes
   - Access to affordable and high-quality medical care
   - Protection from discrimination and stigmatization

2. APPROPRIATE CARE
   - Early diagnosis
   - Access to appropriate medical treatments
   - Support systems for caregivers

3. INCLUSION IN THE UN SYSTEM
   - Full participation of persons living with a rare disease in the work of the United Nations
   - Regular review and monitoring of progress

4. NATIONAL ACTION
   - Commitment from governments
   - Funding and support for research
   - Development of national policies

5. MONITOR PROGRESS
   - Regular reporting on progress
   - Evaluation of outcomes
   - Continuous improvement of policies and practices
New this month!

Video: What is a UN Resolution and why does the global community need it

Watch on YouTube
‘Dear UN’ Campaign: Call for testimonies
‘Dear UN’ Campaign: Call for testimonies

WHY?

• A powerful way to make the UN pay attention to persons living with a rare disease within the SDGs is by sharing stories on everyday life.

• Testimonies show the real experiences and impact on lives, behind the statistics.

• They help policy makers understand and empathise with the challenges individuals, caregivers and families face and they help them see opportunities to respond to them.
‘Dear UN’ Campaign: Call for testimonies

We invite you to share your testimony by completing the short form entitled “Dear UN”, as though you were addressing a letter directly to the UN.

What would you want UN policy makers to know about life with a rare disease?

Share your hopes and expectations!
‘Dear UN’…

The difficulty lies in the impossibility of carrying a routine (...). The problem arises when one day you appear completely healthy, the next day you are sick, and two days later you appear completely normal again. Many people find it difficult to understand the disease and the process”

Woman, Spain

Statement from Rare Barometer Survey: Juggling care and daily life
Illustrative photo. Statement is not linked to the person in this photo
Source: EURORDIS Photo Contest
‘Dear UN’…

I am a wheelchair user and I am not able to work fulltime. I was a researcher but cannot compete with colleagues that work fulltime. I had a limited time contract as a post doctoral researcher and could not get funding after that probably partially due to my diagnose. I felt that employers are "afraid" to employ me, but this was officially never the reason. It took me 8 years to get a new part-time job”

Male, Poland

Statement from Rare Barometer Survey: Juggling care and daily life
Illustrative photo. Statement is not linked to the person in this photo
Source: EURORDIS Photo Contest
‘Dear UN’…

“We have to pay expensive parking fees at hospitals, and have no financial relief, even though paying for child care is very demanding (...). We pay for all the rehab, because it is not provided by health insurance (hippo-therapy, swimming, body workout, rehabilitation). (...)

We pay for everything from our savings and have no support from society”

Woman, Czech Republic

Statement from Rare Barometer Survey: Juggling care and daily life
Illustrative photo. Statement is not linked to the person in this photo
Source: EURORDIS Photo Contest
WHAT TO DO AND WHAT HAS BEEN DONE

YANN LE CAM, RDI ADVOCACY COMMITTEE CHAIR & RDI COUNCIL MEMBER

........................

RDI Membership Meeting
26 May 2021

RARE DISEASES INTERNATIONAL
Next steps towards adoption

November 2020 - January 2021

Civil Society proposal of UN Resolution:
- RDI Advocacy Committee, RDI Council, EURORDIS Board, RDI Members and NGO Committee for RDs

February – April 2021

Campaign events
- Rare Disease Day
  - RDI Policy Event ‘Why a UN Resolution and why now’ (4th March 2021)
- LAUNCH OF THE TOOLKIT (29th April)
- HLPF side-event on the challenges of PLWRD & their families (July 2021)

May – December 2021

Policy event on the final UNGA Resolution (October 2021)

Targeted adoption of Resolution by the UNGA (December 2021)

UN Member States support

Mapping and Meetings of Supportive UN Member States

Core group of Member States:
- Qatar
- Brazil
- Spain

Drafting & Negotiation of zero draft by Core Group (July-Oct)

Increase number of Supportive UN Member state by relevant geographic & economic & political UNGA groups

3rd Committee: Adoption and recommendation to Plenary (Oct-Nov)
Poll – Are You Using Resolution4Rare?

1. Have you visited the Resolution4Rare toolkit page?

2. Have you used the social media images and materials?

3. Have you reached out to a policy maker or UN Representative using the template letter and other advocacy materials?

4. Do you plan to reach out to a policy maker or UN Representative about the call for a UN Resolution?
PANEL DISCUSSION AND Q&A

ROBERTA ANIDO DE PENA, FADEPOF FEDERACIÓN ARGENTINA DE ENFERMEDADES POCO FRECUENTES (FADEPOF)

SAMUEL WIAFE, RARE DISEASE GHANA INITIATIVE

ELIZABETH VROOM, WORLD DUCHENNE ORGANIZATION
Poll – What can we do to help?

1. Do you feel the toolkit has sufficiently prepared you to participate in the call for the UN Resolution?

2. Is the toolkit adapted to your needs, does it meet your needs?

3. Would you need additional tools?

Indicate in the chat what additional tools you may need – for example; a webinar on the topic; one-to-one sessions with RDI staff; translated materials.
Thank you!
GLOBAL
STATE OF THE ART RESOURCE
WHAT IS IT?

An information resource

Needs to be regularly updated, with qualitative data

Needs to be useful to the whole Rare Disease Community worldwide: all stakeholders groups active in the field of rare diseases and OMPs / RD therapies, e.g. patient groups, national authorities and international decision-makers, companies, media, academia, researchers;

- Patient organisations: information and advocacy tool;
- National authorities: reliable information and monitoring tool to inform and shape their strategies;
- For companies: information tool to inform their strategic decision (clinical development and launch)

Resource to become progressively global in a step-wise approach beginning with targeted countries
WHAT IS IT MADE OF?

Three Elements

**The Database:** hosted and managed by Orphanet, publicly available, several national entry points, to be expanded progressively, and provide updated information on national and international policies, activities, services;

**The SoA Reports:** open and publicly accessible, region specific, with information on national strategies, good practices, international initiatives, research, collaboration, policies, new therapies approved, and compassionate use and early access mechanisms;

**The Annual RDI Global Report on Rare Diseases:** "Amnesty International like", a tool to monitor progress on the status of PLWRD in the world, and serve as a reference for RD stakeholders → links with Key Ask 5 of the UN Resolution on Monitoring Progress and implementation
WHY DO WE THINK IT IS USEFUL?

12 years experience with the State of the Art Report in Europe:

- It has been demonstrated that working together with **national authorities**, and having them collaborate with **other stakeholders** to generate this information resource, has **stimulated strategies on rare diseases** at national and regional levels.

- Collaboration in the **Data Committee** (data collection and validation process KEY):
  - National authorities (National Health Systems, Ministries of Health and Research)
  - National Alliances / patient groups
  - Orphanet;

- This approach allows to make **visible** what is invisible - in positive and in negative

- Basis to monitor the **implementation of national strategies** and activities

- Basis to monitor the **implementation of the UN Resolution and the Political Declaration on UHC**

- **Promote key issues** for PLWRD

- **Communication & Awareness** tool
POSSIBLE FRAMEWORK FOR COLLABORATION

Proposed Legal Framework: a Consortium of Partners

- RDI
- EURORDIS
- ORPHANET
- VOZ (project management)

Patient and academic led

Funding proposal

- Donors → Consortium through an educational grant with a Letter of Agreement
- Need to develop a sustainability Model

Moving towards a Social Enterprise approach to create a Public Social Good?
GOVERNANCE STRUCTURE

**Steering Committee**
- Main decision-making body steering the overall project
- Solely led by patient organisations and academia
- VOZ as an observer to separate Governance from Management
- Ensure independence and credibility

**Advisory Committee**
- Multi-stakeholder group of advisors for creative input
- Advise on data collection and donation in different regions, methodology, database structure, etc.
- POs, academia, industry, VOZ, public and private institutions

**Editorial Committee**
- KOLs & experts in RDs (research, clinical, policy, advocacy)
- Invited by the RDI Council with input from the SC
- Support the elaboration of the Annual RDI Report
EXPERIENCE IN EUROPE

CHARLOTTE RODWELL
PARTNERSHIPS, BUSINESS DEVELOPMENT AND STRATEGIC COMMUNICATIONS OFFICER, ORPHANET INSERM
ORPHANEWS ASSOCIATE EDITOR
CO-AUTHOR OF THE REPORT “STATE OF THE ART OF RARE DISEASES ACTIVITIES IN EUROPE”
Global State of the Art Resource – European Perspective

Charlotte Rodwell

*Partnerships, tech transfer & communications officer*

*INSERM US14 – Orphanet, France*

*Rare2030 Partner*

*Co-author of the report « State of the Art of Rare Diseases Activities in Europe »*

charlotte.rodwell@inserm.fr
What is a ‘State of the Art of RD’ resource?

• A tool providing up-to-date, descriptive and visual information pertaining to the RD ecosystem with the aim of promoting exchange of information and experience between countries and stakeholders.

• Covers a wide range of topics of interest for the RD community, at national, regional, and international level.

• Data collected from the all stakeholders/key opinion leaders with the help of established networks.

• All input generated a knowledge base to answer policy and advocacy questions.
Why did we create this resource in Europe?

- Initiative started in 2009 by the European Commission’s Rare Disease Task Force
  - Inspired by the Inventory of Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products (rev. 2005) from the European Commission

- Collect and share experiences on rare disease policies and initiatives at European and Member State level

- Help countries draw inspiration from others to elaborate their National Plans/Strategies for RD (EU Council Recommendation 2009)

- Review progress towards finding a better approach to meeting the needs of the RD patient community, and to inform recommendations for future action at national and EU level
The experience from the European RD community

What is the impact of this resource?

• Valuable and powerful tool for stakeholders, supported by the European Commission in the scope of activities to support RD from 2009 – 2020
  • 15’000 downloads of the report each year*

• Collaborative spirit between stakeholders cultivated

• Informed the construction of national RD plans/strategies and expert group recommendations at European level

• Used to derive indicators to monitor implementation of policies and initiatives

• Fed into a knowledge base used to answer a range of policy and advocacy questions, especially when completed by data from Orphanet
  • Example of use: informing recommendations of the Rare2030 Foresight project

* Example of reports produced by RDTF, EUCERD, ECEGRD 2009-2015
Thank you for your attention!

charlotte.rodwell@inserm.fr

Thanks to my colleague Victoria Hedley (University of Newcastle), who has led the production of this resource since 2015.

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State of the Art tool:
http://www.rd-action.eu/rare-disease-policies-in-europe/

National question bank for example questions:

Rare 2030 Knowledge Base Summaries:
https://www.rare2030.eu/knowledgebase/

Paper based on earlier editions of the report:
Rare disease policies to improve care for patients in Europe (Rodwell & Aymé 2015)
SYNTHESIS & FEEDBACK ON THE GLOBAL SOA RESOURCE SURVEY
55 Participants From 31 Countries Joined the Global SoA Resource Webinar

19 Participants From 7 Regions Completed the Global SoA Resource Survey
Would you be open to taking part in an Advisory Committee for the Global State of the Art Resource?

- Yes: 53%
- Not Sure Yet: 47%
DATA COLLECTION AND VALIDATION

Would you be willing to support Data Collection and Validation from your country to help build the Global SoA Resource?

- Yes: 64%
- Not Sure Yet: 36%
THE THREE ELEMENTS

Rank the proposed Global State of the Art Resource elements in order of importance (1-MOST Important to 3-LEAST Important)

1. SoA Reports
2. SoA Database
3. The Annual RDI Global Report on Rare Diseases
### On Which Areas Should the Proposed Annual RDI Global Report Focus?

<table>
<thead>
<tr>
<th>Area</th>
<th>Focus Count</th>
</tr>
</thead>
<tbody>
<tr>
<td>International Rare Disease Collaborations and Initiatives</td>
<td>14</td>
</tr>
<tr>
<td>International Policy and the United Nations</td>
<td>11</td>
</tr>
<tr>
<td>Rare Disease National Plans around the World</td>
<td>10</td>
</tr>
<tr>
<td>Low and Middle Income Countries</td>
<td>9</td>
</tr>
<tr>
<td>Rare Disease National Alliances and Federations Around the World</td>
<td>8</td>
</tr>
<tr>
<td>Approved Therapies</td>
<td>7</td>
</tr>
<tr>
<td>Access Mechanisms and Compassionate Use Programs</td>
<td>6</td>
</tr>
<tr>
<td>Specific Disease or Therapeutic Areas</td>
<td>5</td>
</tr>
<tr>
<td>Data Return and Connection</td>
<td>1</td>
</tr>
</tbody>
</table>
Collaboration with all stakeholders to ensure the data is accessible and sustainable

What other funders can we reach out to beyond pharmaceutical companies → Sustainability Model

Understand and address the reasons for lack of interest to support the Global SoA resource

Think about the different regulatory constraints and how to address them in different regions and countries
NEXT STEPS

April to May
GATHER INFORMATION FROM ALL RDI MEMBERS, FUNDERS AND ADVISORS

By May
REACH OUT TO POTENTIAL FUNDERS FOR CONFIRMATION ON FUNDING

By July
CONFIRM THE MODEL AND SECURE FUNDING COMMITMENTS

Aug
BUILD STRUCTURE FOR REPORT WITH RDI MEMBERS, EURORDIS, ORPHANET

By Nov
IMPLEMENT REPORT CHANGES AND DATA COLLECTION STANDARDS

Jan
EXECUTE PLAN TO PUBLISH REPORT IN Q2 2022
Global Network Concept Model

Are we going in the right direction?

RDI Programme Team
RDI Membership Meeting, 26 May 2021
Vision

People living with a rare disease no matter where they live can reach a network of expertise to access appropriate diagnosis, care and treatment

SDG Agenda 2030

- Strengthen healthcare systems
- Break down barriers accessing care:
  - open and direct self referral to RD Hubs
  - healthcare pathways

UHC Implementation

Cover 2 billion people with hubs in major cities ➔ 85 million people living with a rare disease

- Expedite and provide accurate diagnosis:
  - scaling up of diagnostic capacities
  - reduce misdiagnosis
  - diagnosis in < 1 year after 1st contact with a doctor
Nationally Endorsement of Single or Multi-Centres as a National Hub & applying to join a Regional Hub
Mandated national role to strengthen health systems’ competency in Rare Diseases
Internationally recognized as a ‘lighthouse’ for rare disease
National Population & RD Coverage (%)
One National Hub per Country (<50M pop.)
Knowledge ‘adaptors’ for national healthcare systems to access global knowledge

Virtual multi-centre regional network (10-20 National Hubs) & National Patient Organisations
Joint Application to demonstrate equality and inclusive approach
Configuration defined locally
Scale up diagnostic capacities of the National Hubs and connect to an Undiagnosed Disease Programme
Connected to Orphanet Information Hub
Provide the infrastructure and platform to supports collaboration

Network of Regional Hubs (x20-25 Hubs) & International Federations
WHO Secretariate, Steering Committee & External Advisory Committee
Disease Cluster Working Groups to offer expert advice and coordinate ‘community actions’
Advise on Global strategy and guidelines e.g.: WHO roadmap for rare diseases
Capacities to share data, information, practice and exploitation of information to build global knowledge
Exploit advancements in technology
Pilot & Scaling Up

Pilot Phase (2022-25)
- As a minimum Pilot to include one Regional Hub per Region (between 6-8 Regional Hubs)
- Each Regional Hub is formed by between 4-6 National Hubs & National Patient Organisations
- Selected number of PO Members to take part in Pilot
- Test Network’s Governance Model
- Initiate 2-3 ‘Disease Cluster’ Working Groups

Scaling Up (2025-30)
- Expand the number of National Hubs in the pilot Regional Hubs (up to x20)
- Applications for the establishment of new Regional Hubs (total x25)
- Invitations for new PO Member Application
- 6-8 Global “Disease Cluster” Working Groups
Creating Relationships by forming the Panel of Experts

The Panel of Experts – more than 220 patient representatives and clinicians - will advise on the following areas:

- Advise on technical aspects of the development of the WHO Collaborative Global Network for Rare Diseases
- Provide validation of the technical research findings
- Inform development of the networks model (scope, structure and functions) to meet the needs of all patients irrespective of location
- Provide a bridge to other relevant initiatives in order to create synergies

<table>
<thead>
<tr>
<th>WHO Region</th>
<th>No. Experts</th>
</tr>
</thead>
<tbody>
<tr>
<td>Africa region</td>
<td>34</td>
</tr>
<tr>
<td>Region of the Americas</td>
<td>58</td>
</tr>
<tr>
<td>East Mediterranean region</td>
<td>32</td>
</tr>
<tr>
<td>European region</td>
<td>40</td>
</tr>
<tr>
<td>South East Asia region</td>
<td>15</td>
</tr>
<tr>
<td>Western Pacific region</td>
<td>49</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td><strong>228</strong></td>
</tr>
</tbody>
</table>

- **Participate in 4 online workshops through 2021/22**
  - 1st Round of Online Workshops held in March 2021
  - 2nd Round of Online Workshops scheduled in June 2021
  - 3rd Round of Online Workshops scheduled in October 2021
  - 4th Round of Online Workshops scheduled in Q1 2022
Connecting to the Global Population

Email: matt.johnson@rarediseasesint.org
WHO Collaborative Global Network 4 Rare Diseases

We are already, progressively building the Global Network through engagement with the global rare disease community:

Connecting People
- Engaged the International Federations and Patient Groups
- Connecting with 83.4% of the global population, from 101 countries (51.8%).
- Through this connection of the rare disease community together, the foundation of the Global Network already exist!

Creating Relationships
- Building on this foundation and connect with experts from around the work.
- Build a partnership between with the rare disease population under an Panel of Experts (>190 experts).
- Extend this partnership to include national authorities to secure political support.

Cooperative Action
- Co-creating the Global Network and securing ownership from all four corners of the world!
- Building the Global Network from grass roots formalising the connection between the experts (in the Panel of Experts)
- Pilot will build on the collaboration and engagement of experts (patients and clinicians) from each of the WHO regions
Panel of Experts (2021/22)

1st Workshop:
- Start creating a regional collaboration
- Feedback on patient needs (Global & Regional)
- Co-creation of the Concept Model

2nd Workshop: Core Principles
- Mapping potential members (National)
- Partnering to form a regional HUB (Regional Cooperation)
- Feedback of Disease Clustering Proposal (Global Collaboration)

3rd Workshop:
- Co-design readiness appraisal prototype (based on core principles)
- Complete readiness appraisal with Potential Members

4th Workshop:
- Prepare for Pilot Phase & partner with national authorities secure support and endorsement

Q1 2021  Q2 2021  Q3 2021  Q1 2022
Thank you
GLOBAL ACCESS: ESSENTIAL RARE DISEASE MEDICINES, BARRIERS & STRATEGIES

MEMBERSHIP MEETING
26 MAY 2021
WHY ACCESS STRATEGY IS ESSENTIAL

ONLY SMALL FRACTION OF PATIENTS IN ANY COUNTRY HAVE ACCESS TO APPROVED ORPHAN DRUGS

- 8% of global population is affected by a rare disease
- <5% of rare diseases have a treatment
- <10% of RD patients in USA receive orphan drug approved for their indication
- <1% of RD patients worldwide have access to orphan drugs
Treatment Access Working Group

IRDiRC : International Rare Disease Research Consortium
Established in 2011; Members: 64 organizations (as of May 2021)
**Mission**: Unites national and international governmental and non-profit funding bodies, companies, umbrella patient advocacy organizations, and scientific researchers to promote international collaboration and advance rare diseases research worldwide

**Vision (2017-2027)**: Enable all people living with a rare disease to receive an accurate diagnosis, care, and available therapy within one year of coming to medical attention

**PACC**: Patient Advocacy Constituent Committee (PACC) established in 2017 to increase patient input. Chair: Durhane Wong-Rieger (CORD/RDI)

**Rare Disease Treatment Access Working Group (2020) with three aims:**
1. To improve standards of care for RD patients by promoting access to approved medicines
2. To initiate research into barriers to accessing RD medicines, especially in LMICs
3. To define opportunities to address those barriers

http://www.irdirc.org/
RDTA WORKING GROUP PRE-PUBLICATION
(Publication Pending—Revisions Submitted)

Essential List of Medicinal Products for Rare Diseases – Recommendations from the IRDiRC Rare Disease Treatment Access Working Group

William A. Gahl
NIH: National Institutes of Health

Durhane Wong-Rieger
Canadian Organization for Rare Disorders

Virginie Hivert
EURORDIS Rare Diseases Europe

Rachel Yang
China Alliance for Rare Diseases

Galliano Zanello (galliano.zanello@inserm.fr)
Institut national de la santé et de la recherche médicale: INSERM https://orcid.org/0000-0001-8512-4384

Stephen Groft
NIH: National Institutes of Health

Online in February 2021

UPDATE 204 medicines extracted and organized in seven disease categories

Living document to be regularly updated
RDTA WG ACTION PLAN

Three Research to Action Phases: focus on lack of access in LMICs and inequitable access in high-income countries

1\textsuperscript{st} Paper: list of medicines considered essential for rare diseases (FDA, EME, WHO Essential Lists, China NMPA)

2\textsuperscript{nd} Paper: barriers to access stratified by types of therapy, characteristics of rare disease populations, and key country parameters (investment in health, health systems capabilities, rare disease priorities, etc).

3\textsuperscript{rd} Paper will consider strategies for improving access targeted towards the identified barriers.

RDTA WG List of Medicines considered essential for Rare Diseases based on approvals by key regulatory agencies (US, EU, China) plus WHO Essential Lists

- Databases of medicines designated as OMPs or approved for Rare Disease indications;
- WHO Model Lists of Essential Medicines and of Essential Medicines for Children

Collated List of 204 Essential Medicinal Products: efficacious, safe and have significant impact on quality or duration of life.

Organised into seven disease categories as initial iteration of a “living document” to be updated periodically.
CHALLENGES ALONG ACCESS PATHWAY IN REAL-WORLD SETTINGS
Transform Health System to Transform Treatment Access

**POLICY, HEALTHCARE CAPACITY, FUNDING & MANAGEMENT**

Advocating for increasing policy awareness, policy guidance, as well as sufficient budgeting and funding support of patients & families

**INFLUENCING PATIENT JOURNEY**

<table>
<thead>
<tr>
<th>AWARENESS</th>
<th>SCREENING &amp; DIAGNOSIS</th>
<th>TREATMENT</th>
<th>AFTER CARE</th>
</tr>
</thead>
<tbody>
<tr>
<td>Educating patients, families, community workers about prevention, disease, symptoms and treatment</td>
<td>Supporting healthcare workers to help increase awareness, availability and effectiveness of screening and diagnosis</td>
<td>Providing healthcare workers with the skills, tools and access to preventive and therapeutic medicines to give the best possible care</td>
<td>Educating patient, families, healthcare workers on long-term follow-up, disease management care, travel support and after care</td>
</tr>
</tbody>
</table>

**ADDRESS NATIONAL & LOCAL CHALLENGES**

- E.g., Lack of diagnostic, screening capabilities & capacities
- E.g., Lack of financial means
- E.g., Physical access barriers
- E.g., Lack of support for patient duties at home

**WHAT ARE SYSTEM-WIDE GAPS?**

**WHAT ARE DISEASE-SPECIFIC BARRIERS?**
Transition to RDI - Next Steps

- Use Collated List collated to stimulate interactions among patient organisations, healthcare providers, industry and government agencies to improve standards of care and promote access to treatments.

- Integrate IRDiRC RDTA Working Group within RDI and align activities with priorities of RDI and Members;

- RDI is recruiting a Programme Manager to prioritise access position and activities to improve/accelerate diagnosis and access to care and therapies.

- Capacity-building to enable Members to use WHO Essential Medicines List & In-Vitro Diagnostics List; apply for inclusion to the Essential Lists.

- Launch and expand Expert Faculty to grow knowledge base and support capacity-building activities on access.
THANK YOU