



# Towards a WHO Collaborative Global Network for Rare Diseases: Needs Assessment and Concept development

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RDI Global Meeting 2020

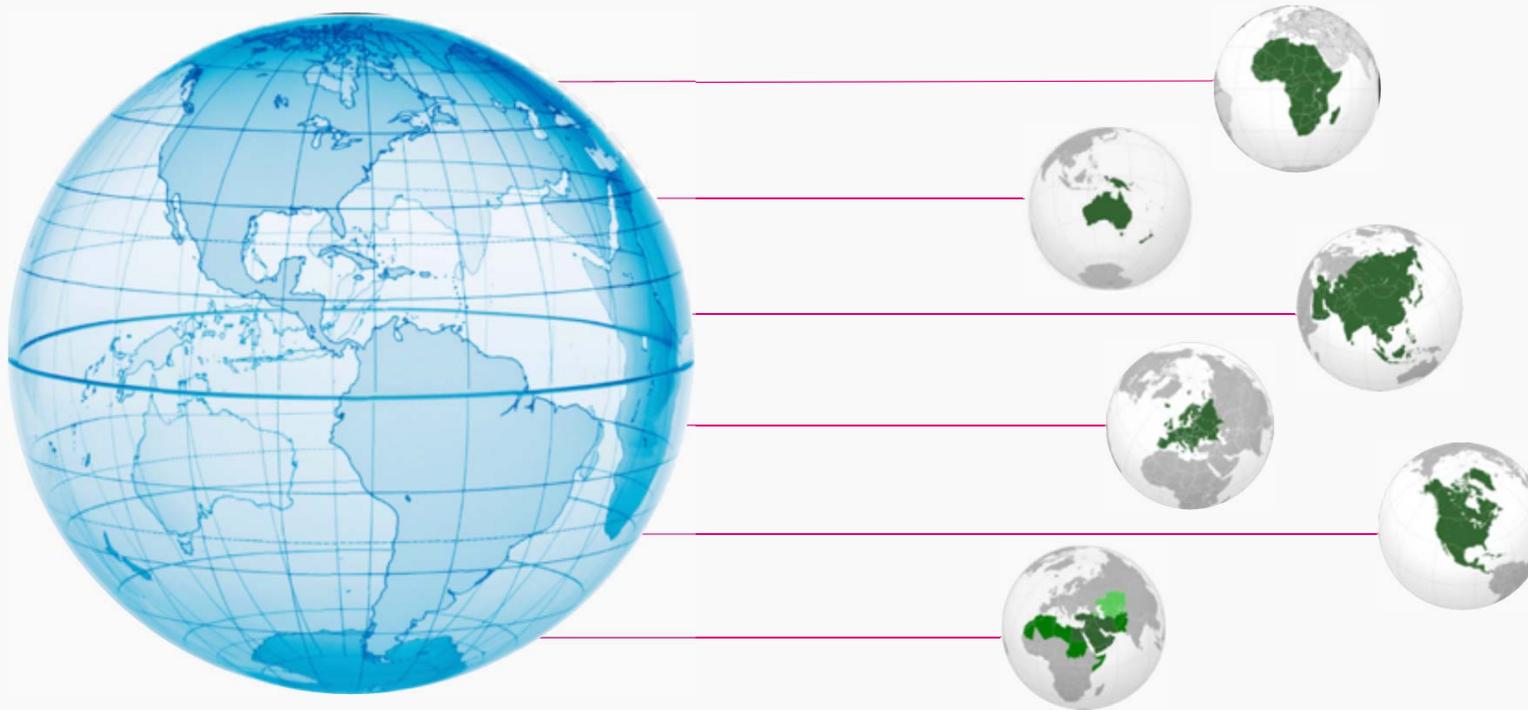
19th May 2020 (15:15-15:30)

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# Global Network Model



**WHO Collaborative Global Network for Rare Diseases**

**+**

**Global RD Hub Members**  
(connected into 6 Regional Networks per WHO Regions)

Source: If you need to add a source or footnotes to a slide you can do so here



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# Guiding Principles

## Strategic goals:

Structure grouping of rare diseases under thematic areas (under “*global programmes of care*”), inclusive of all rare diseases, leaving no one behind.

*Global RD Hubs* will be internationally University Medical Centres recognised in the field of rare disease, who provide healthcare, research, education and technology

Selection and identification of Global RD Hubs will be through endorsement of Member States and an independent, *volunteer international accreditation system*

## Applying to the WHO Collaborative Global Network for Rare Diseases, Global RD Hubs commit to:

- Locally, to improving access to and coordination of high quality diagnosis, care and treatment and support the development of local of health systems capacity and competency in rare diseases
- Globally, to collaborate with other Global RD Hubs, to provide access expertise and healthcare; gather expertise and generate knowledge, through clinical guidelines and education; exchange data (to FAIR standards) and conduct research activities.



# CGN International Collaboration

## Collaborate



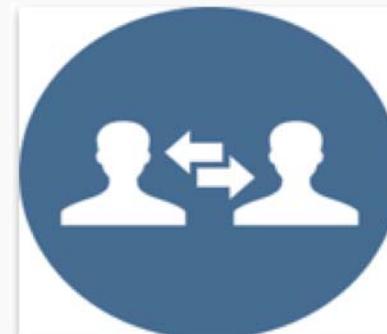
- Technology as an enabler to connect and share knowledge and best practices
- Sharing networking experiences to improve networks capacities

## Coordination



- Virtual healthcare and advice
- Platform and tools to improve timely diagnosis
- Research and Clinical trials
- Registering data in common registries

## Continuity



- Strengthen healthcare competency in RD
- Training and education – twinning programme
- Development evidence base and clinical guidelines

# WHO CGN4RD - Key Components



- Global RD Hub
- Affiliated Member

● Advocate member

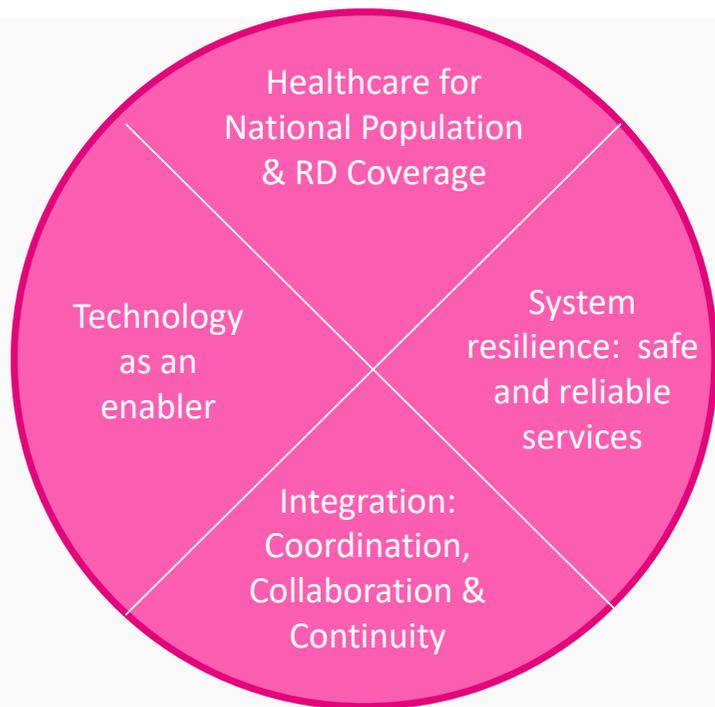
- Global Network
- Structured under 6 WHO Regions



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# Global RD Hub Model

## RD Hub Member



National Endorsement of Global RD Hub under National Rare Disease Plan

### Global RD Hub National Footprint:

- Spearheading access to timely diagnosis and quality care
- Strengthening local health systems competency in RD

## Global Network

### Primary Aims

- Coordinated Global Action to deliver Universal Health Coverage
- Experts offer acute advice and are virtually present at the bedside and at home
- Share knowledge and expertise to increase evidence base
- Top-notch research and world renowned training centres

### Members

- Global RD Hubs – (single vs multi-centre)
- Affiliated Partners
- Advocate Members

### Programmes of Care

- Thematic grouping
- Action plan
- Multi-sector partner Interface & resources



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# RDI Members Feedback

Feedback Summary	Our Actions
National policy important to promote for RD and UHC	<ul style="list-style-type: none"> <li>• Endorsement of Global RD Hubs to be under a national policy that set the expectation and obligation of the hub to strengthen the NHS competency and provide national coverage.</li> </ul>
Centres are mostly in capitals with uneven distribution of care	<ul style="list-style-type: none"> <li>• National endorsement will be based on a centres ability to collaborate with the wider health system to provide national coverage.</li> </ul>
What constitutes a Global RD Hub (disease-specific vs majority RD)? How to ensure country are not left outside, without an expert centre?	<ul style="list-style-type: none"> <li>• Define Global RD Hub as a connector of centres and networks (single or virtual multi-centre).</li> <li>• Expanded type of membership – from Global Hubs and Affiliated Partner - to enable participation.</li> </ul>
Access to care and education are highest priority areas	<ul style="list-style-type: none"> <li>• WHO CGN4RD main objective to 'spearhead access to diagnosis and quality care. The Network will also be based on the principles of learning health systems with education being a core functions.</li> </ul>
Promote multi-disciplinary working and breaking down professional silos	<ul style="list-style-type: none"> <li>• Eligibility standards for a Global RD Hub will include multi-disciplinary working.</li> </ul>
Important to bridge the gap between paediatric and adult networks for care	<ul style="list-style-type: none"> <li>• Express the notion of 'programs of care' throughout the life journey.</li> </ul>
Need for communication material in other languages to secure buy-in	<ul style="list-style-type: none"> <li>• Development of a 'Promotional Kit' in some different languages</li> </ul>



Tell us about your potential  
Global RD Hub?



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# Advocate Members

## Advocates Requirements to Join

- Advocates representing national / international population
- Recognised competency in advocates
- Endorsement by Patient Organisation Board
- Assessment by independent assessors



## Role in WHO Global Network

- Experts by experience in rare diseases
- Support vulnerable and isolated populations to access care
- Understand the needs of the rare disease community
- Capacity building and promotion health literacy
- Bridge between the Network and the community



# Poll

What should be the key activities / functions of a global - please select your top three highest priorities:

1. Develop rare disease competency in national health system
2. Make visible experts and sign-post patients
3. Support the development of an 'official' recognition of rare disease
4. Experts offer acute advice and organisation of care in patient crisis
5. Develop diagnostic tools and best practice
6. Collection data and benchmark outcomes of care
7. Exploit advancements in technology and innovation
8. Develop new best practice and innovation (through knowledge sharing)
9. Offer training and education activities
10. Support clinical research activities



# Project Overview & Timeline



# Project Overview

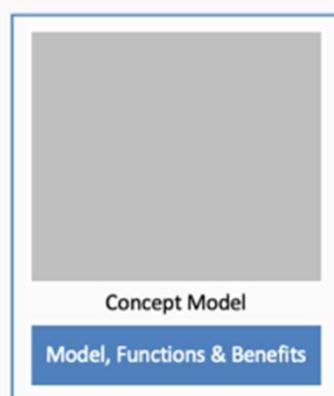
## Top-down Research: Needs Assessment Study:

- Literature Review to identification of evidence base for networking
- Needs Assessment to identify population profile and needs
- Semi-structured interviews – insights from existing networks

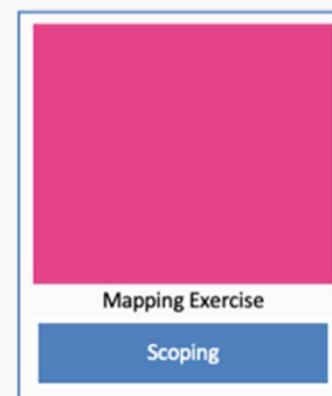
## Deliverable: Methodological Framework

- Eligibility criteria, application forms and supporting tools
- Assessment process, criteria and standards

## Discovery Phase (2020)



## Development Phase (2021)



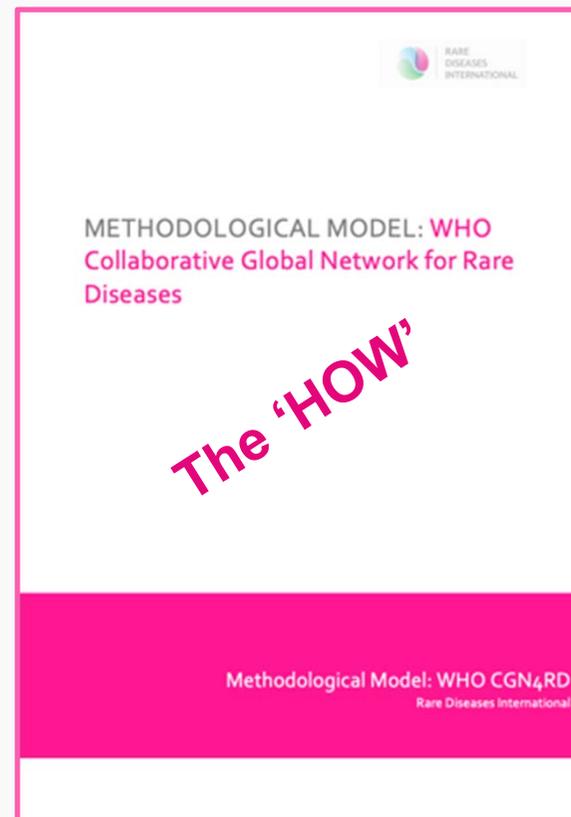
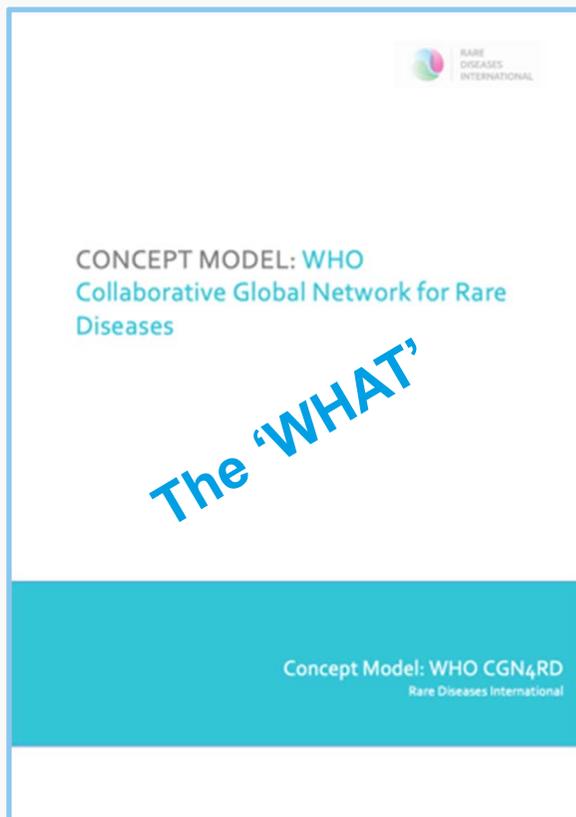
## Deliverable: Concept Model

- Network governance, funding and value proposition
- Global RD Hub model, structure and functions

## Bottom-up Research: Mapping Exercise:

- Mapping Exercise of potential Global RD Hubs
- Readiness Assessment of potential Hubs interview (remotely)
- Exhaustive review of existing assessment approaches

# Project Output



# Project Methodology

**Survey of Patient Needs (2020):**  
Reference Cases representing a  
'cluster of RD'



## Example:

- Duchenne Muscular Dystrophy identified as a reference case for 'neuromusculoskeletal and movement-related functions'
- Collaborate with the World Duchenne (International Federation) to survey their Members / Patients from the different WHO Regions

**Balanced Scorecard:**  
Used to baseline competency  
of potential Hubs in site visits



## Example:

- Peking University Hospital (China), etc... assess to join WHO GCN4RD using the balance scorecard
- Hospitals need to demonstrate how they coordinate care to meet the holistic needs of people living with a RD
- Demonstrate being a national 'lighthouse' expert centre

**Assessment Methodology (2021):**  
Used to assess potential Hubs for pilot



# Needs Clustered by Function, Disability & Health



Voice and speech functions; Structures involved in voice and speech



Mental functions and structures of the nervous system



Functions and structures of the digestive, metabolic and endocrine systems



Genitourinary and reproductive functions and structures



Sensory functions and pain; the eye, ear and related structures



Functions and structures of the cardiovascular, haematological, immunological and respiratory systems

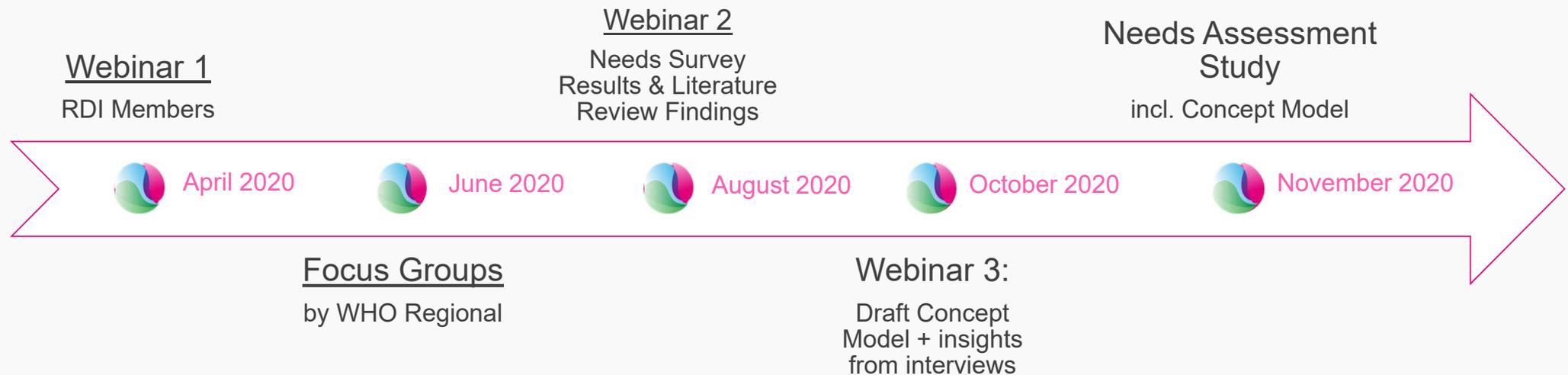


Neuromusculoskeletal and movement-related functions and structures



Functions of the skin and related structures

# Engagement with RDI Members, 2020



## Also in 2020:

- Survey of patient needs for a number of 'reference cases' rare diseases
- Interviews of existing Networks (including Patient Lead, Clinical Lead and National Authority Lead)
- Planning assumption c. 10 existing Networks

## In 2021:

- Site visits of existing Centre of Experts (with National Alliance Lead)
- Planning assumption 3 Centres per WHO Region



