WHO Collaborative Global Network 4 Rare Diseases

RDI Advocacy Committee Webinar

27th April 2020

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Webinar Format

Webinar Format:

- Part 1: Introduction

- Part 2: Outline Concept Model for the WHO CGN4RD + Q&A

- Part 3: Project + Q&A

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Part 1:
Introduction

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WHO, RDI & Rare Diseases

WHO and Rare Diseases:

- WHO Director General Statement on Rare Diseases, February 2018

- UN Political Declaration on Universal Health Coverage includes Rare Diseases, September 2019, and, WHO is responsible for the implementation of UHC

- Memorandum of Understanding (MoU) signed between WHO and RDI, December 2019

The Study Specific Objective (MoU)

"WHO and RDI will collaborate to improve the organisation of health care services by proposing to establish, as appropriate, a global network of multidisciplined specialised expert centres for rare diseases, in a structure approach grouping rare diseases by therapeutic areas, so to be inclusive of all rare diseases, to leave no one behind."

"WHO and RDI will prepare a need assessment study so to propose the conceptual and methodological framework for the network with a strategic, bottom up approach that promotes the identification and support of highly specialised multi-disciplinary centres of expertise, connects centres of expertise regionally and internationally into WHO collaborative global networks for rare diseases while encouraging local capacities to generate additional expertise"

Dr Rüdiger Krech, WHO Director, Health Promotion,
Division of Universal Health Care / Healthier Populations

https://www.youtube.com/watch?v=ap0iM3LZfqQ

Vision

WHO Collaborative Global Network for Rare Diseases (WHO CGN4RD) connect major university research hospital centres (hubs) with multi-disciplinary rare disease specialist capacities, at a regional and global levels.

This initiative contributes to improve access to universal healthcare coverage world-wide and to strengthen healthcare systems within current available resources.

Within the SGDs Agenda 2030, the ambition is to make this Global Network potentially available to serve 2 billion people with hubs in major cities (hence about 85 million people with a rare disease), specifically:

- To expedite and provide accurate diagnosis, reducing the time to diagnosis and the misdiagnosis, hence addressing the diagnostic odyssey to less than one year after the first contact with a medical doctor (a common objective of RDI, EURORDIS, IRDiRC, Global Commission Diagnostic Odyssey)

- To break down the traditional barriers in accessing care often experienced by patients and their families, through open, direct self-referral to the Network's Global RD Hubs as much as through potential healthcare pathways

- To access holistic, affordable and quality highly specialised healthcare for diagnostic, care and prevention of co-morbidities

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Part 2: Outline Concept of the WHO CGN4RD

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Characteristics & Profile of RD

Low prevalence cases

- Small patient populations, scattered across large geography
- World-wide population between 263-446M
- 98% population affected by <400 rare diseases
- 2% affected by 6,000 RD (1 in 1M prevalence)

Based on 68% of prevalent RD based on EU definition (<50/100,000), data from literature.

Estimating global point prevalence of rare diseases: analysis of the Orphanet database. BMC Public Health, submitted

Increased complexity

- Complex multi-system, chronic, progressive, disabling and degenerative
- Over 6000 rare diseases
- 72% RD are genetically based
- 25% people wait up to 30 years for diagnosis
- Poor outcomes and in some cases reduced life expectancy

Limited expertise & evidence

- Lack of visibility and recognition of experts
- Clinical outcomes improve in higher volume clinical teams
- Centralisation of care and knowledge leads to increased outcomes, but politically sensitive

Ineffective treatments

- Lack of effective treatments and available therapies (95% population)
- High-cost interventions with limited access to adequate care
- Maintain clinically safe and financially sustainable
- Increasing costs and high investment required in a competitive market

Refences:
- 'Giving existence to people living with a rare disease in health systems - Naming, counting, taking action'. Prof. Ana Rath, Orphanet, Inserm US14, France (NGO Committee RD, 2019)
- Hentschker, C., Mennicken, R., 2018. The Volume–Outcome Relationship Revisited: Practice Indeed Makes Perfect. Health Serv. Res. 53, 15–34. https://doi.org/10.1111/1475-6773.12696
- Morche, J., Mathes, T., Pieper, D., 2016. Relationship between surgeon volume and outcomes: a systematic review of systematic reviews. Syst. Rev. 5, 204. https://doi.org/10.1186/s13643-016-0376-4

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Poll 1

Poll 1: For your rare disease and your country, what is the biggest area of unmet need?

- Lack of a structured healthcare system

- Lack official recognition of rare disease

- No expert centre for rare diseases

- Not knowning where to find the experts

- Barriers to accessing expertise due to funding

- Nearest expert centre is abroad

- High cost of treatment

- Clinical research is fragmented

- Lack of innovation

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What is a Global Network for RD…? How will it benefit my disease, my country…?

Global Network Model Structured under WHO Regions

WHO Collaborative Global Network for Rare Diseases

+

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

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WHO CGN4RD - Key Components
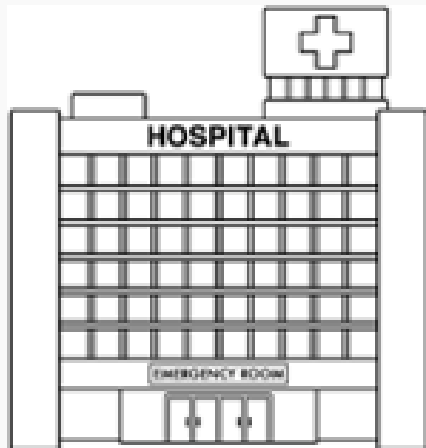
🔵 RD Hub & Affiliated Member

🔵 Advocate member

🔵 Global Network

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Global RD Hubs

Nationally Endorsed Hospital:

- Internationally recognized Expert Centre of rare disease
- National Population Coverage
- Providing healthcare, research, education and technology
- Majority of Rare Diseases Disease Coverage (%)

Children's Hospital,
Vancouver

Sick Kids Hospital,
Toronto

Children's Hospital,
Montreal
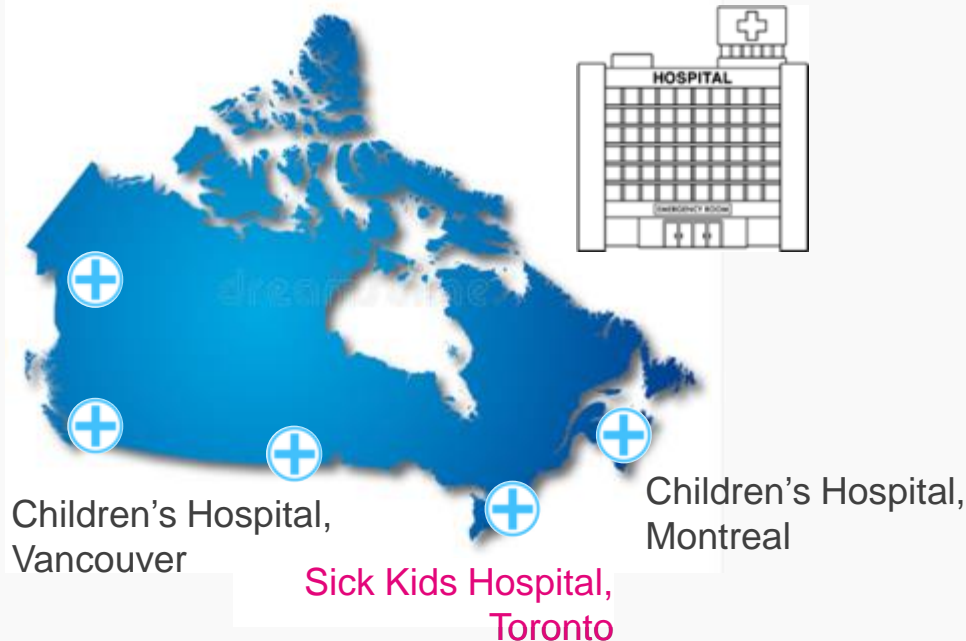
Independent assessment & international accreditation:

- Population and disease coverage
- Coordinated patient centred-care
- Collaboration and continuty
- System resilence
- Use technology as an enabler

Main Function of Global RD Hub:

- Improve access to and coordination of high quality diagnosis, care and treatment
- Support the development of local of health systems capacity and competency in rare diseases

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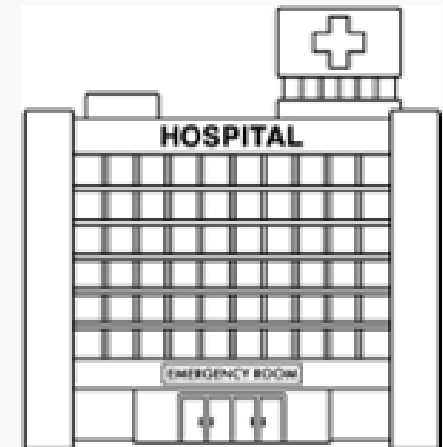
Poll (2)

Poll 2: When thinking about your disease area and country, does your local expert centre meet this vision for a Global RD Hub?

Is your healthcare system well organised in how they provide rare disease services?

- Excellent: Highly developed system with national networks for rare disease centres

- Good: Good level of national centres for some rare diseases

- Average: Some expert centres nationally recognised as experts in the field of rare diseases

- Minimal: Expert clinicians active internationally but with no national recognition

- Basic: Basic care

Please let us know the name of your centre who maybe be a potential RD Hub in the chat!

Global RD Hub vs Affiliated Member

Nationally Endorsed Hospital:

- Internationally recognized Expert Centre of rare disease

- National Population Coverage

- Providing healthcare, research, education and technology

- % Disease Coverage
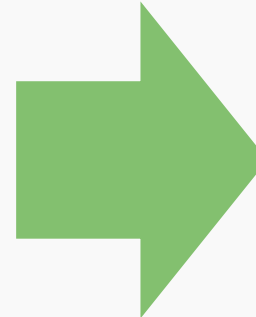
Institute of Human Genetics & Newborn Screening Reference Center, Maynila

Independent assessment & international accreditation:

- All Rare Diseases vs Single Disease

- Coordinated patient centred-care

- Collaboration and continuty

- System resilence

- Use technology as an enabler

Main Function of Global RD Hub:

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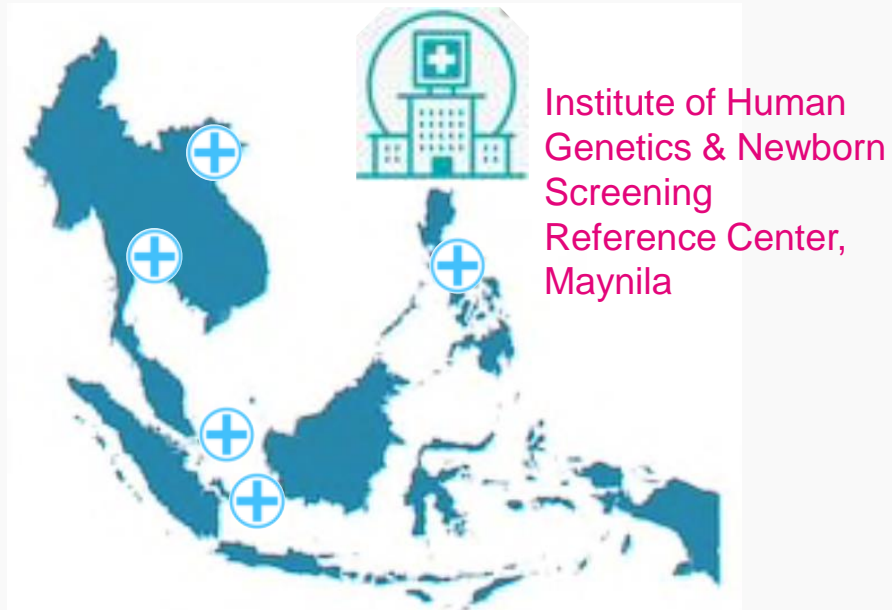
Advocate Members

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

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WHO Collaborative Global Network 4 RD

Function:

- Experts offer acute advice and organisation of care in patient crisis
- Virtually present at the bedside and at home
- 'Top-notch' research players
- World renowned training centres offering training opportunities
- Exploit advancements in technology and innovation

Global Network Activities:

- Structured activities under "*Programmes of Care*"
- Clustering of rare diseases
- Share expertise and knowledge
- Collaborate on research, education and healthcare

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Poll (3)

Poll 3: What should be the key activities / functions of a global

- Experts offer acute advice and organisation of care in patient crisis

- Virtually present at the bedside and at home

- 'Top-notch' research players

- World renowned training centres offering training opportunities

- Exploit advancements in technology and innovation

- Reduce the time it takes to diagnosis of a rare diseases

- Improve access and quality of diagnosis, care and treatment

- Decrease the impact (and number) of rarer diseases through (maybe) finding cures.

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Incentives

Political

- National recognition and political support
- Unique partnership model - Ministry of Health, National Alliance and Research Institute
- Strengthening political relationships
- International recognition and visibility - WHO CGN4RD brand – leading for UHC & SDG3

Financial

- Exploration to national, regional and international funding mechanisms
- Structured interface with third sector partners - research, clinical trials, genomics, digital and IT development and innovation

Healthcare

- Increased cases and coverage (incl. income associated with care)
- Increase in safe and sustainable services and increase in service knowledge / competency of centres
- Building rare disease competency locally
- National leadership role
- Increase access for vulnerable populations

Collaboration

- International collaboration –
- Learning and codification of new knowledge
- Data sharing and registries
- Research groups
- Access to new technology and innovations

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Any Questions?

Part 3:
Project

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Project Governance

World Health Organisation
(With Division UHC & Healthccare Systems)

Needs Assessment Study

Rare Diseases International
(with EURORDIS and other members support)

Memorandum of Understanding

• Operational Description of RDs and prevalence / incidence data

• Global Network's Needs Assessment of Conceptual & Methodological Framework (Deadline: Nov. 2020)

Health Standards Organisation (HSO)

(supported by RDI / EURORDIS)

+ Project Team + Advisory Board

Conduct Needs Assessment, Literature Review, Mapping Exercise etc.

External Advisory Board

Technical Proposal on the Needs Assessment Study

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External Advisory Board

The main purpose of this External Advisory Body is to:

- Ensure continued independent input on the activities and results from the perspective of the main stakeholders

- Provide advice on the development of the conceptual model for the WHO CGN4RD and methodological framework for assessment the RD Hub Members;

- will provide continuity from the *Discovery Phase* to the *Development Phase*

External Advisory Body experts will be invited x2 representatives from the key stakeholder groups

Total of 16 Members (plus two Project Team leads).

Project Overview & Timeline

Discovery Phase

Development Phase

2022

2023

Sustainability Phase

| Needs Assessment Study | Methodological Framework | Assessment | Pilot | Sustainability Phase |

2020

2021

Deployment Phase

2024 - onwards

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Work Package A & B

Network Perspective

Top-down approach

The Concept Model describes the 'WHAT':

- Identification of the characteristics, structure and services of the Networks

- Defines the added value, outcomes, Impact and success factors

- Based on the population needs, insights gained from existing networks and published evidence

The 'WHAT'

Work Package A

NA Study & Concept Model

Health Framework

incl. Needs-Based Scorecard

The 'HOW'

Work Package B

Methodological Framework
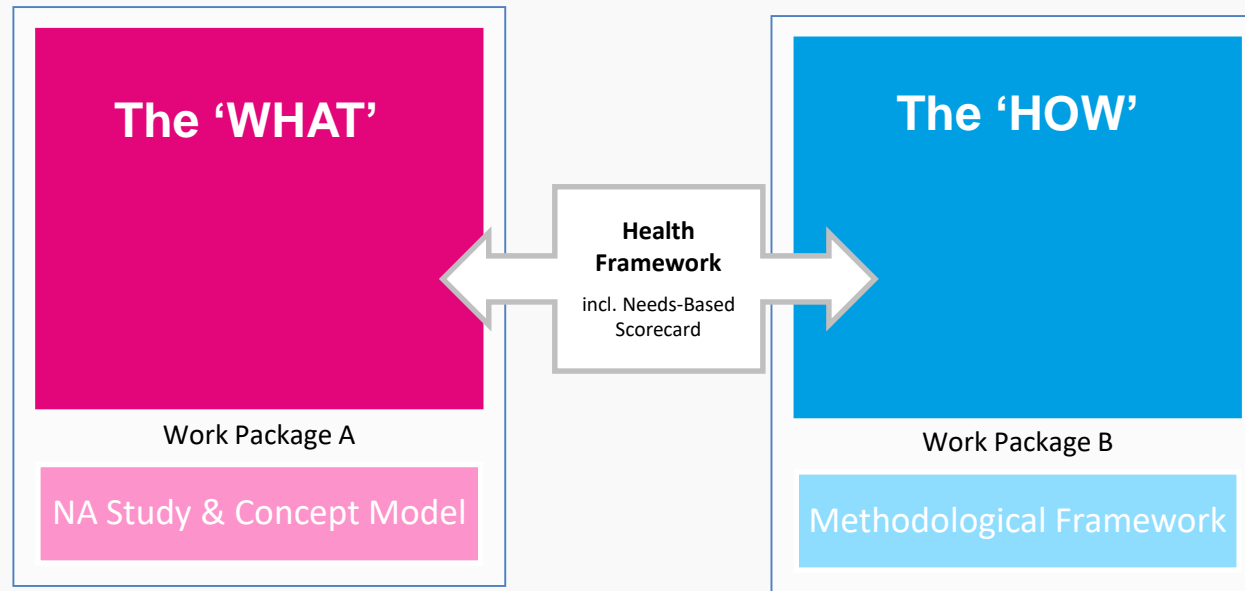
Hospital Perspective

Bottom-Up Approach

The Methodological Framework describes the 'HOW':

- Application process and eligibility criteria to join the Network

- Assessment model based on existing best practice

- Assessment process and standards to assess hospitals competency

- Based on a baseline of hospitals competency to meet the patient needs – functionality, disability and health needs

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Network Perspective (WP1 – 2020)

Task A.1 Survey of Patient Needs

- Clustering of rare diseases

- Identification of 'Reference Cases' to be surveyed with International Federations

- Profile of patient needs for representative populations

- Development of a 'scorecard' of patient needs (function, disability and health)

Task A.2 Semi-structured Interviews

- Review of existing Networks

- Interviews with three representatives per Network – patient lead, clinical lead and national authority

Triangulate insights gains on the added value, impact and benefit of Network

Identification the characteristics, structure and functions

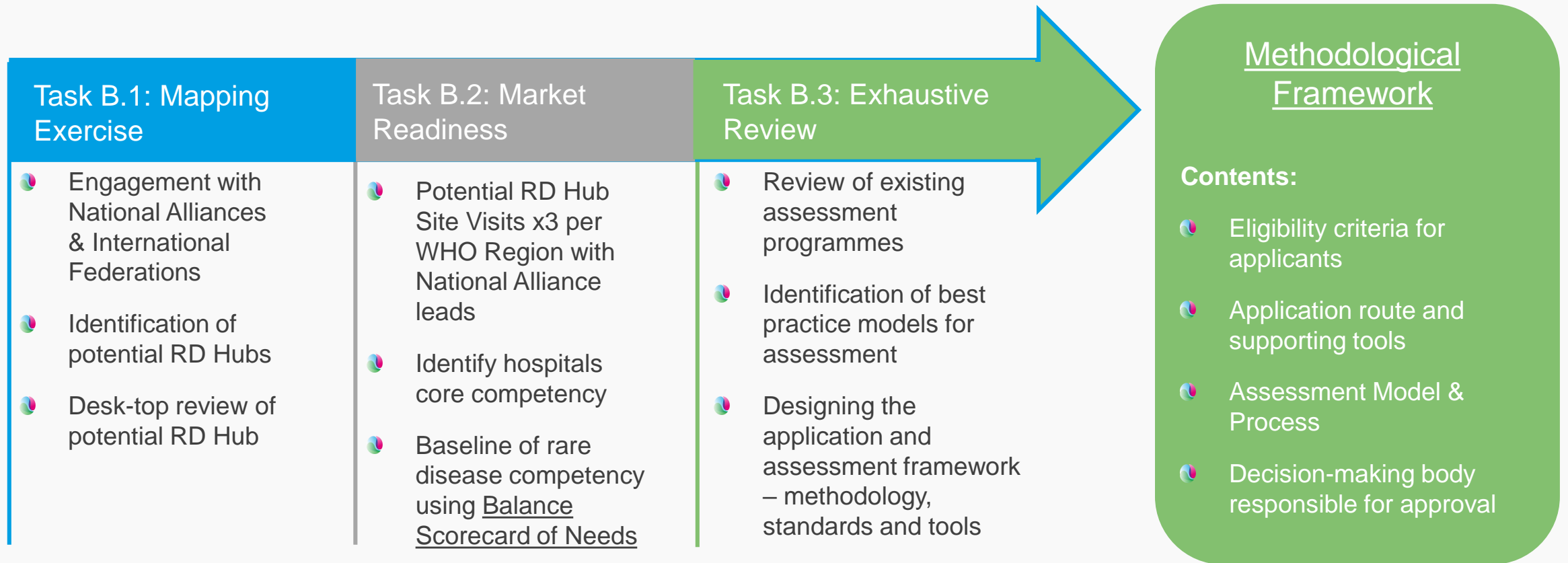**Task A.3 Literature Review**

- Evidence base on the added value of networking

- Identification of characteristics and functions

Needs Assessment Study

Contents:

- Population Needs for people with a rare diseases

- Summary of evidence base and insights gained for Networking

- Concept Model of the WHO GCN4RD and its Members

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Hospital Perspective (WPB – 2021)

Task B.1: Mapping Exercise

- Engagement with National Alliances & International Federations
- Identification of potential RD Hubs
- Desk-top review of potential RD Hub

Task B.2: Market Readiness

- Potential RD Hub Site Visits x3 per WHO Region with National Alliance leads
- Identify hospitals core competency
- Baseline of rare disease competency using Balance Scorecard of Needs

Task B.3: Exhaustive Review

- Review of existing assessment programmes
- Identification of best practice models for assessment
- Designing the application and assessment framework – methodology, standards and tools

Methodological Framework

Contents:

- Eligibility criteria for applicants
- Application route and supporting tools
- Assessment Model & Process
- Decision-making body responsible for approval

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Poll 4

Poll 4: What do you consider being the WHO Global Networks (and its RD Hub Members) biggest impact for your disease, in your country?

- Help to structure the healthcare system in your country

- Support the development of an 'official' recognition of rare disease

- Support the development of expert centre for rare diseases

- Identify where the experts are in your country

- Support access to expertise (and reduce the costs of care

- Enable advice for care to local centres

- Enable access to treatment

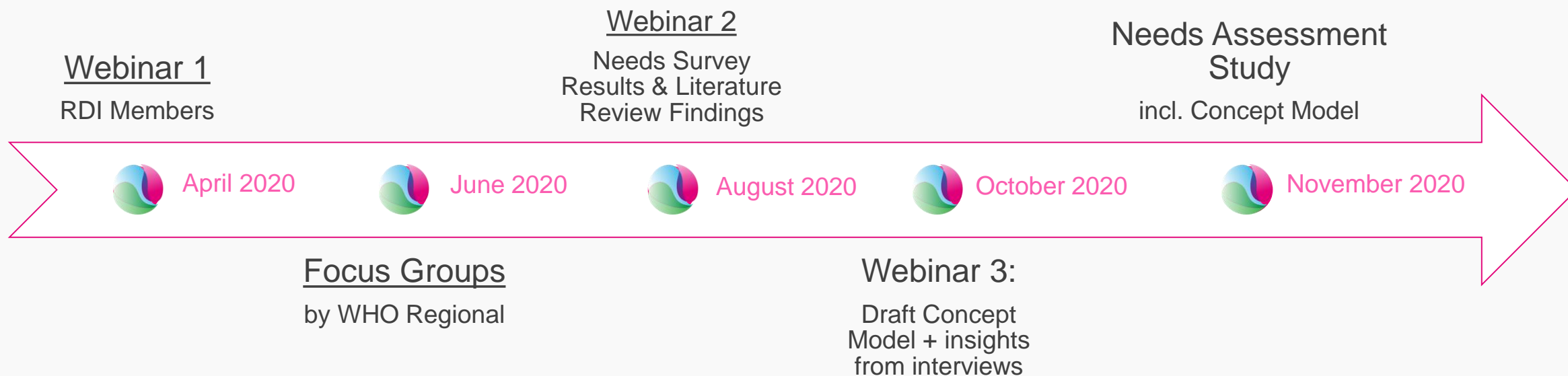- Support clinical research activities

- Enable access to innovation

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Any Questions?

Next Steps...

Engagement with RDI Members in 2020

<u>Webinar 1</u>

RDI Members

<u>Webinar 2</u>

Needs Survey
Results & Literature
Review Findings

Needs Assessment
Study

incl. Concept Model

April 2020   June 2020   August 2020   October 2020   November 2020

<u>Focus Groups</u>

by WHO Regional

Webinar 3:

Draft Concept
Model + insights
from interviews

Also in 2020:

- 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

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