



European Reference Networks: the WHAT, the WHY and the WHEN

**Rare Disease – Denmark: Workshop
on European Reference Networks
(ERNs)**

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Rare Disease Day 2020

- 13th edition on 29 February 2020
- Thousands of awareness-raising events held in 100 countries!
- Brings together patients, families, patient organisations, healthcare professionals, industry, institutions & policy-makers worldwide to raise awareness.



The WHY: Characteristics & Profile of RD

- Small patient populations, scattered across large geography
- Critical Mass of 263-446M world-wide (3.5-5.9% preval.)
- 98% population affected by <400 rare diseases
- 2% affected by 6,000 RD

Low prevalence cases



*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

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

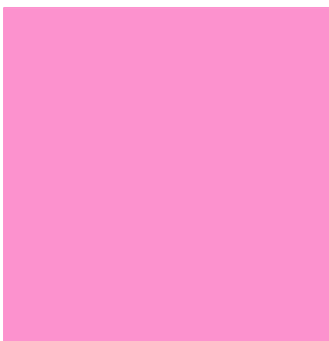


Increased complexity



- Complex multi-system, progressive, disabling & degenerative diseases
- >6000 RD heterogeneous in nature, but share common characteristics
- Limited access to adequate care
- Poor outcomes

Ineffective treatments



- Maintain safe and sustainable services – clinical competency and financial viability
- Lack of effective treatments
- High-cost interventions and requiring high investment

The WHAT: ERN Spearhead EU Expertise



Networks harness the collective knowledge and experience of experts, focusing on a common goal to drive **improve access to diagnosis and treatment** by enabling expertise, not the patient:

- Connect expert, anchored into national health systems
- Creating a critical mass of cases and data
- Inclusive, not exclusive, multi-professional, not single professional
- Common language, common platform enabling mobility of expertise
- Collaborative spirit, trust and sharing with a common goal
- Drive research and new therapies
- Increase patient outcomes and survival

Types of ERN Membership

- **Full HCP Member:** Open to all expert centres that meets the specific criteria for expertise in a specific RD
- **Affiliated Partners:** Member State with **without a Member of a Network** may decide to designate healthcare providers with a special link to a given Network.
- **Patient Representatives:** Open to all patient representatives (EURORDIS members & non-members)

Full Member

HCP Member

- 'Endorsed' by their MS under their legislation
- EC Competency
- Complete a Full Application and independent EC Assessment
- Role explicitly defined in the EC ERN Delegated Acts

Affiliated Partner

National Associate Centre

- MS Competency – national designation process
- Clinicians, hospitals, laboratories, ...
- No formal definition
- No defined designation process in MSs

National Coordination Hub

- MS Competency through a designation process
- Coordination and information role
- No formal definition
- No defined designation process in MSs

ePAG Patient Representatives

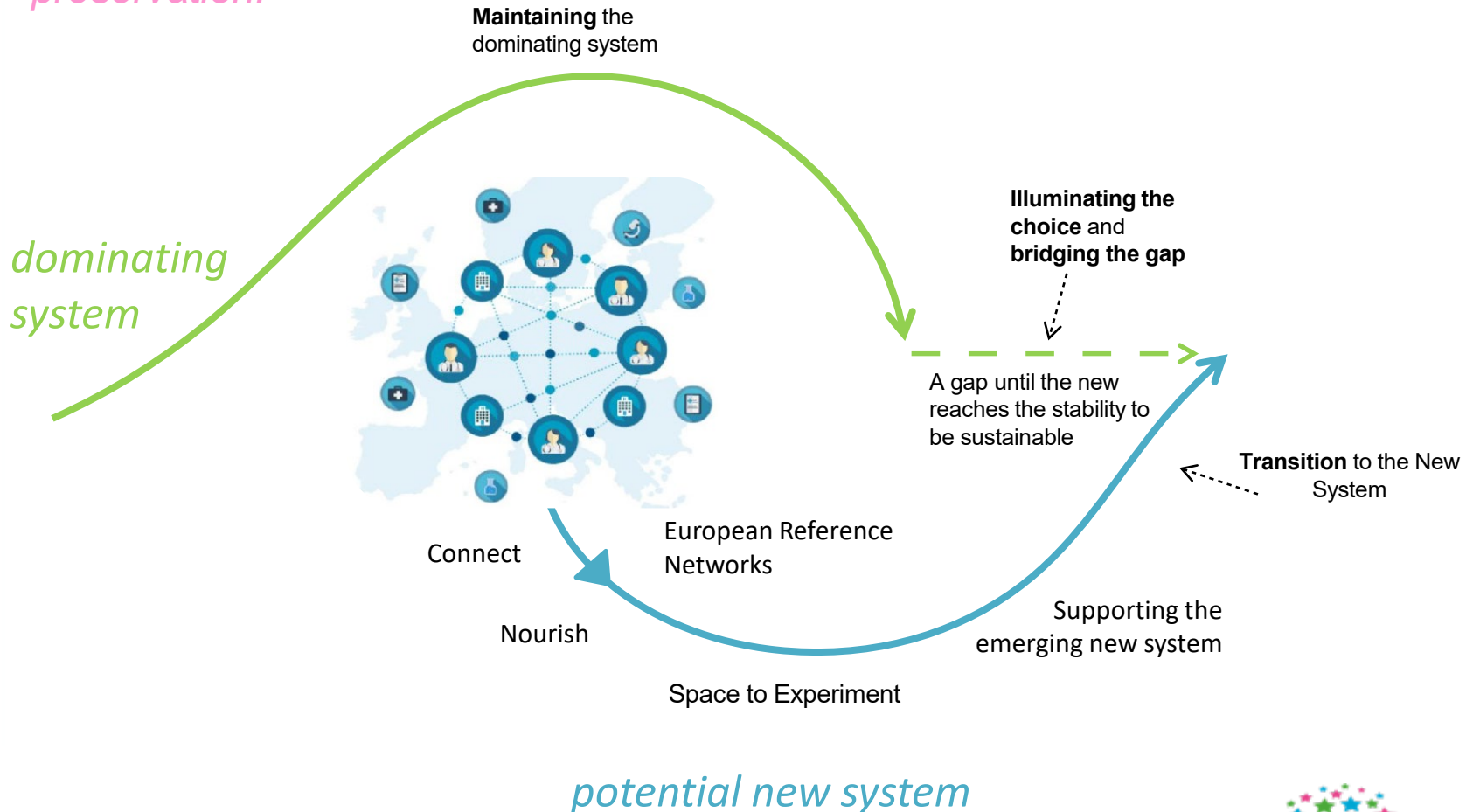
All Patient Representatives

- Invited members, not legal members
- EUCERD Recommendation
- EURORDIS lead the development of common framework to optimize patient involvement
- Creation of 24 ePAG Groups in 24 ERNs

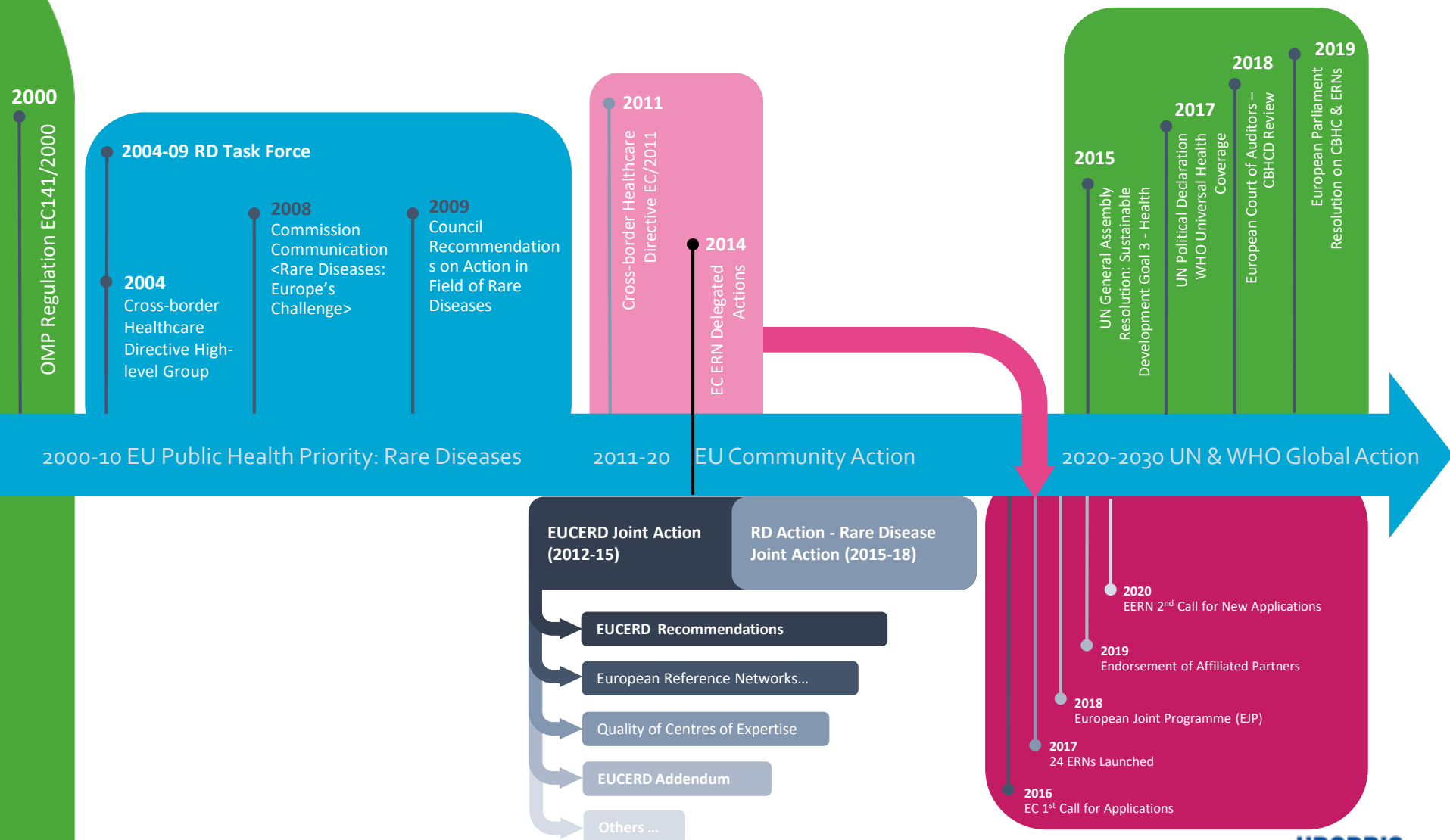
Paradigm Shift in Healthcare for RD

from MDT to 'Networked Care'

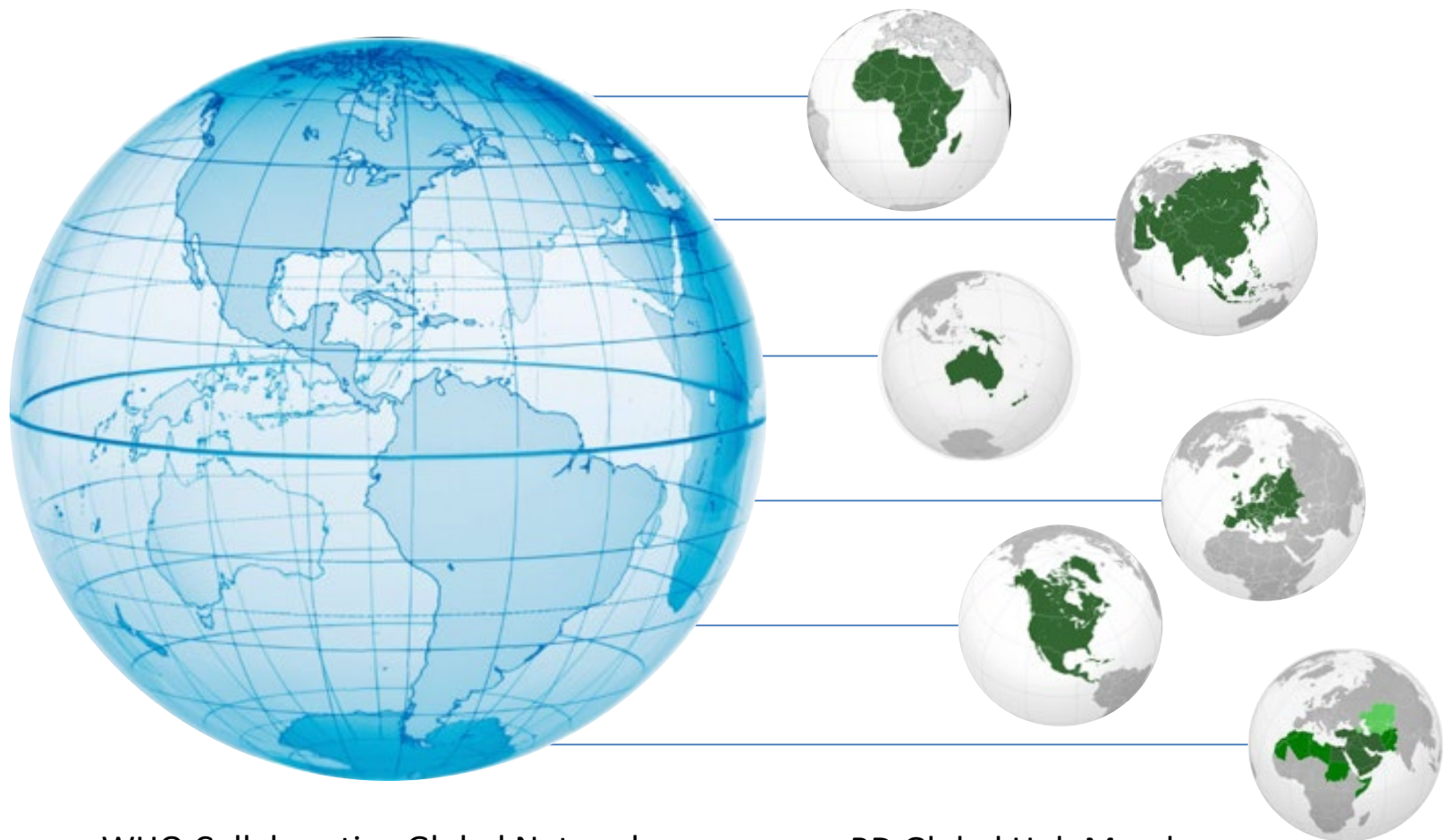
A system is inclined to self preservation.



The WHEN: 20 Years of creating the opportunity ERNs offer



The FUTURE: WHO Global Collaborative Global Networks for Rare Diseases



WHO Collaborative Global Networks
for Rare Diseases

+

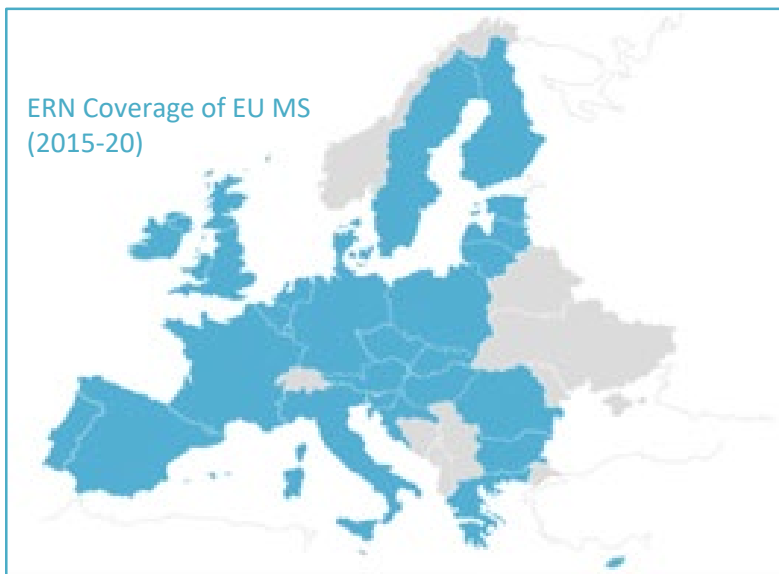
RD Global Hub Members
(connecting 6 WHO Regions)

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Shift from Coverage to ERN & NHS Integration

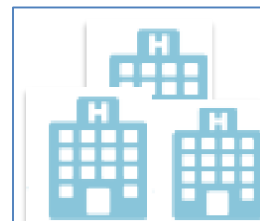
ERN Coverage of EU MS
(2015-20)



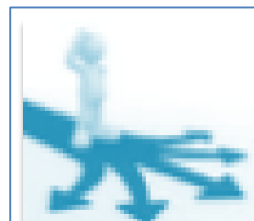
Yr		Total	Coverage
2015-16	Application		
2017	Full Members	900	44%
	Member States in All ERNS	3	12.5%
2019	Affiliated Partners	250	74%
	Member States in All ERNS	10	42%
2020	Full Member Applications	850	>95%
2020	ERN RD Expansion	-	+53 RD



ERN Agenda shift to focus on Integration of ERNs
into NHS (2020-25)



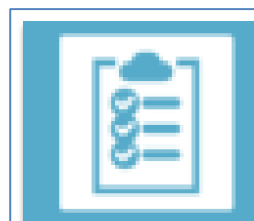
National Networks



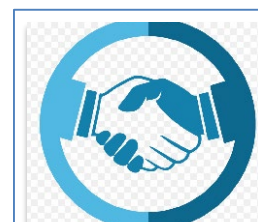
Referral Pathway



Care Pathways



ERN Information



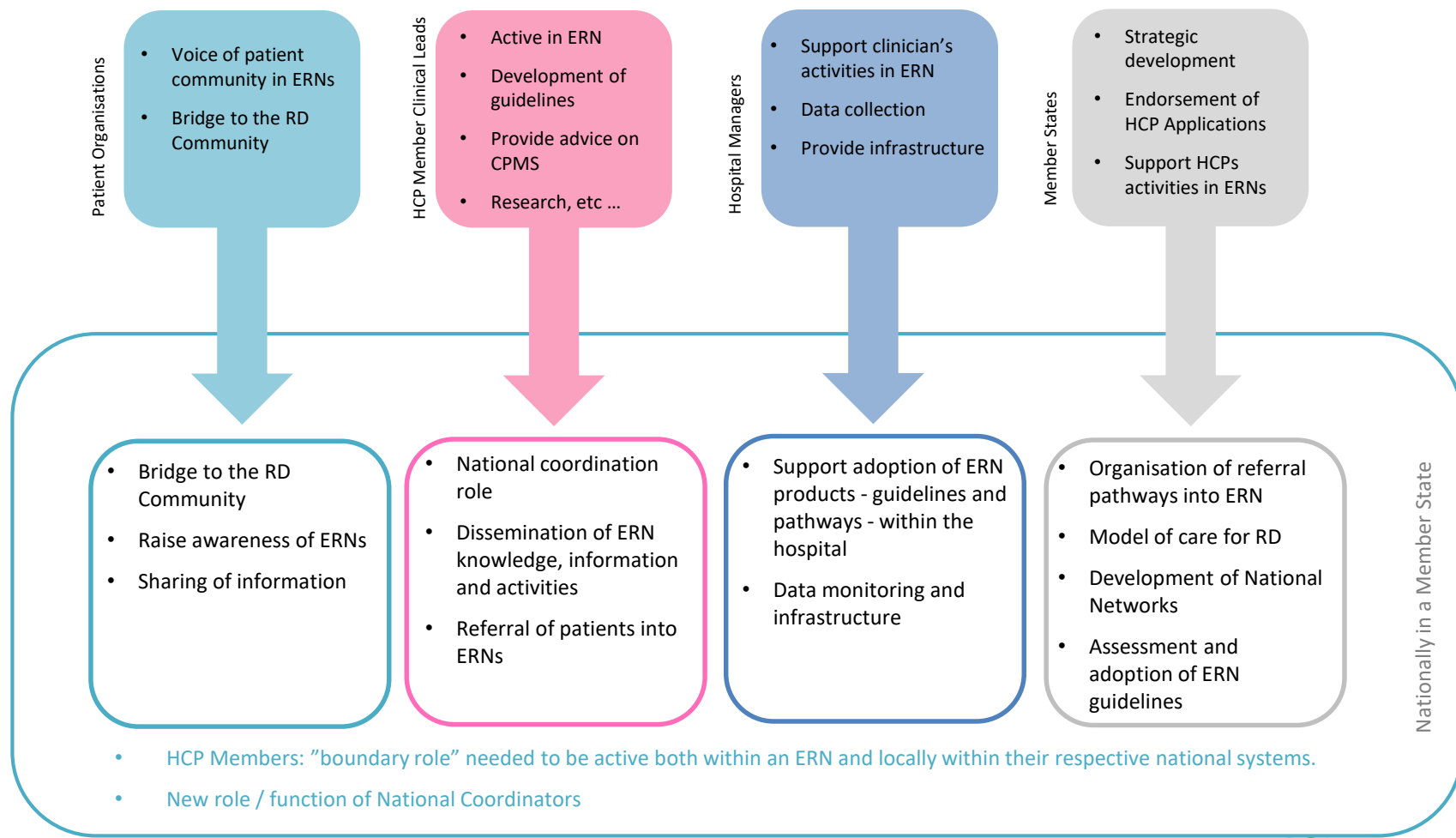
Member State Support

NB: National Coordination Hubs - designated by Malta (Mater Dei Hospital), Luxembourg (Centre Hospitalier du Luxembourg) and Hungary (Semmelweis University).

What are the National Needs ... in Denmark?

1. What are Denmark's healthcare needs specific to rare diseases and highly specialised healthcare?
2. How can ERNs support and complement the Danish healthcare system:
 - For rare disease patient community ...
 - For expert teams, primary care practitioners (etc...) ...
 - For hospital services ...
 - For Regional and National Authorities.

ERNs (and HCP Members) Role at a National Level



National Networks (Rec. 1)



Denmark

Representative in the ERN Board of Member States:

Søren NEERMARK, Danish Health Authority

Members:

Aarhus University Hospital

Endo-ERN
ERN LUNG
ERN eUROGEN
EURACAN

Copenhagen University Hospital Rigshospitalet

Endo-ERN
ERN EYE
ERN LUNG
ERN eUROGEN
ERN GUARD-HEART
MetabERN
ERN PaedCan
ERN RARE-LIVER
ERNICA

Odense University Hospital

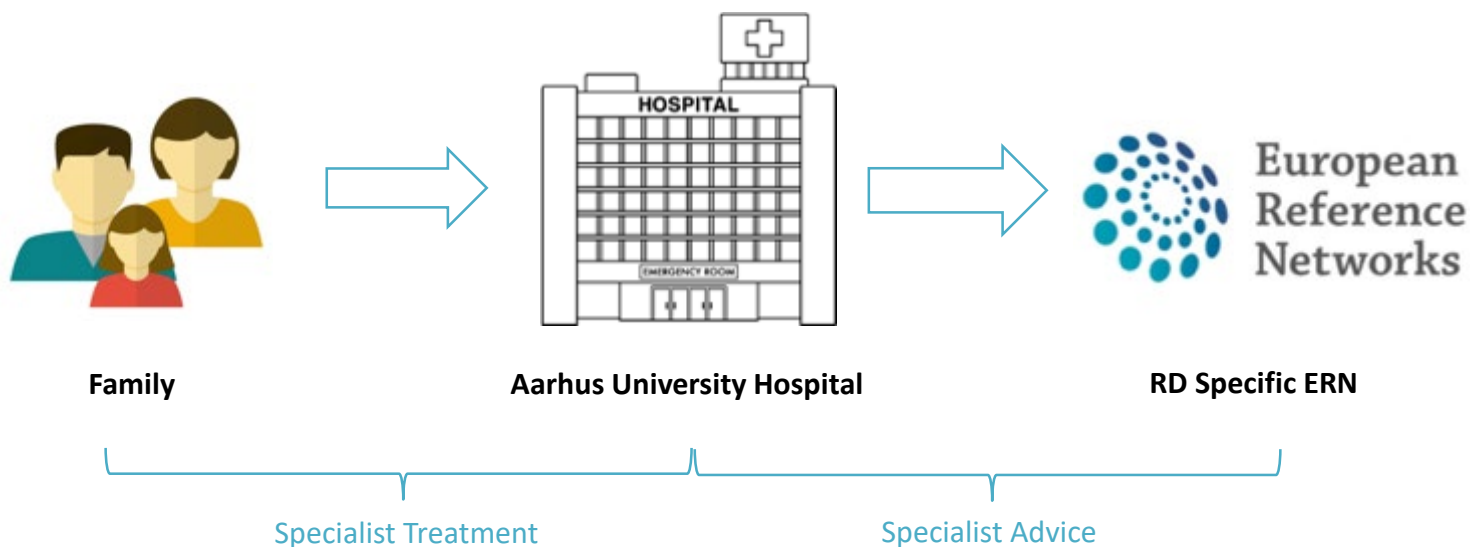
ERN Skin
ERNICA
VASCERN

Zealand University Hospital

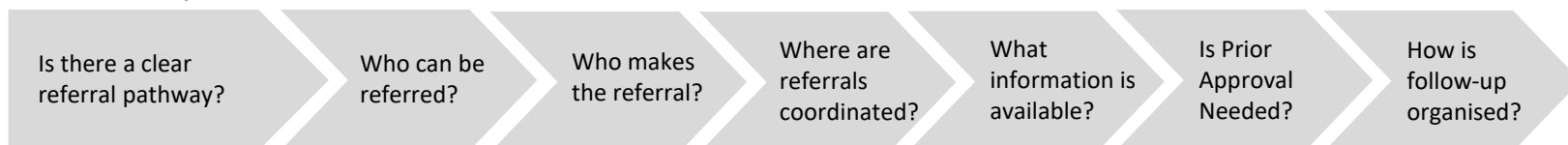
ERN Skin

Referral Pathways (Rec. 2)

Virtual Consultation - Expertise travels, not the Patient



Referral Pathway



End of 2020, patients benefit from the ERN via the CPMS:
903 panels opened & 428 panels closed

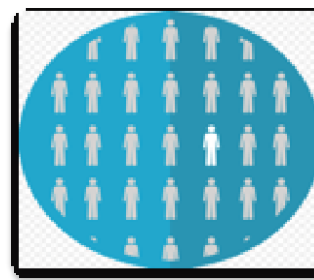
ERN Information (Rec. 3)



Guidelines &
Consensus Statements



New innovation &
emerging Best Practice



Registries



Virtual Healthcare



Research



Digital Pathways



Patient Information



Education & Training
Webinars

- Raise awareness of ERN and leverage the national benefits of networking
- Dissemination of new information and knowledge
- Identify legal and policy developments needed in implement ERN activities

Care Pathways (Rec. 4)

Top-Down

- Review of ERN care pathways and how they can be implemented nationally, assessing impact on care coordination



Disease specific national care pathways

Bottom-Up

- Service mapping and their alignment with the new Network Care Pathways
- Benchmark national pathways on the needs of patients – service improvement within hospitals and between hospitals
- Development of national care pathways – disease specific, aligned with the ERN recommended structure.

Member State Support (Rec. 5)



- Denmark (probably) will have 50 Health Care Professionals involved in the ERNs by the end of 2020.
- How can expert teams be supported (financial, resources, administrative) to be active within the ERN as well as within Denmark?
- Good will is not enough and will quickly evaporate and is not a sustainable model
- Expert teams need capacity to act and contribute in a meaningful way
- Support experts to be active at a national level, within Denmark and with the ERNs
- Identify hospital resources and infrastructure that can support their experts within the hospital, nationally and within the ERN(s)

Key Messages



1. ERNs are more than the sum of their parts.
2. Investment into this opportunity will unlock the potential of networking for the local population needs.
3. Leading to improvement in the quality of life and reduce burden on individuals, their families and on the healthcare system.
4. Once in a lifetime opportunity for the rare disease community, locally within Denmark.
5. Opportunity to drive clinical efficacy, service efficiency and reduce waste.
6. Opportunity for multi-sector stakeholders and partners to have a structured interface to collaborate.
7. Give this opportunity a real chance of delivering and being successful.
8. Now, need to focus on leveraging the benefits of ERNs within each Member State ...

Tak for din opmærksomhed



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RARE is many

RARE is strong

RARE is proud!



