ERN DEPLOYMENT 2017/18

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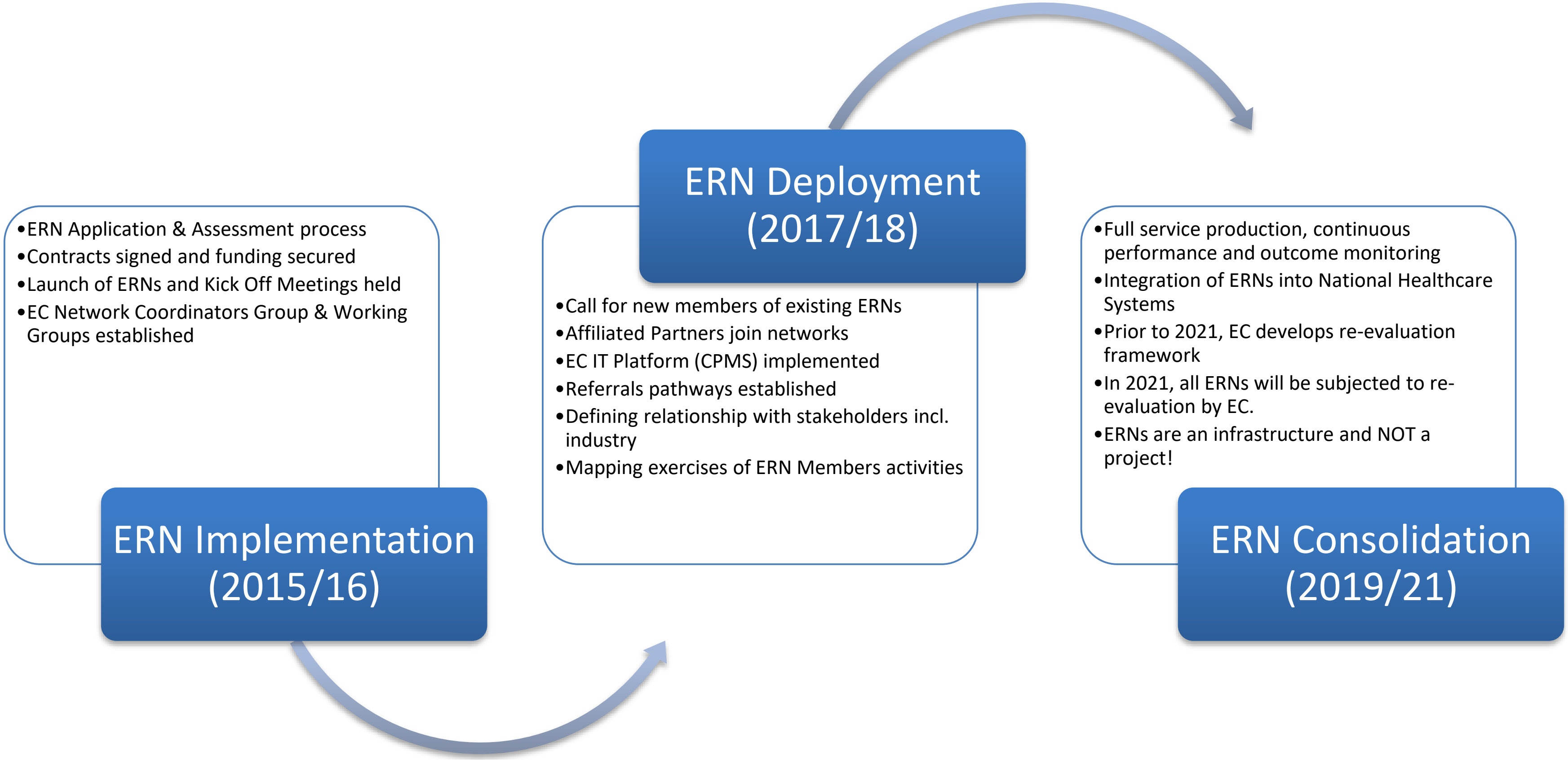
ERN Development & Implementation Cycle



ERN Implementation (2015/16)

- ERN Application & Assessment process
- Contracts signed and funding secured
- Launch of ERNs and Kick Off Meetings held
- EC Network Coordinators Group & Working Groups established

ERN Deployment (2017/18)

- Call for new members of existing ERNs
- Affiliated Partners join networks
- EC IT Platform (CPMS) implemented
- Referrals pathways established
- Defining relationship with stakeholders incl. industry
- Mapping exercises of ERN Members activities

ERN Consolidation (2019/21)

- Full service production, continuous performance and outcome monitoring
- Integration of ERNs into National Healthcare Systems
- Prior to 2021, EC develops re-evaluation framework
- In 2021, all ERNs will be subjected to re-evaluation by EC.
- ERNs are an infrastructure and NOT a project!

ERNs Deployment (2017/18)

European Reference Networks (ERNs)

- “A network connecting providers of highly specialised healthcare (‘CEs’) in domains where patients and expertise are scarce”
- Concept grounded in ‘Cross-Border Healthcare Directive’ - supported by legal acts
- **Expertise travels across borders, rather than the patient**
- Originated through patient advocacy
- Success is due to a major multi-stakeholder effort

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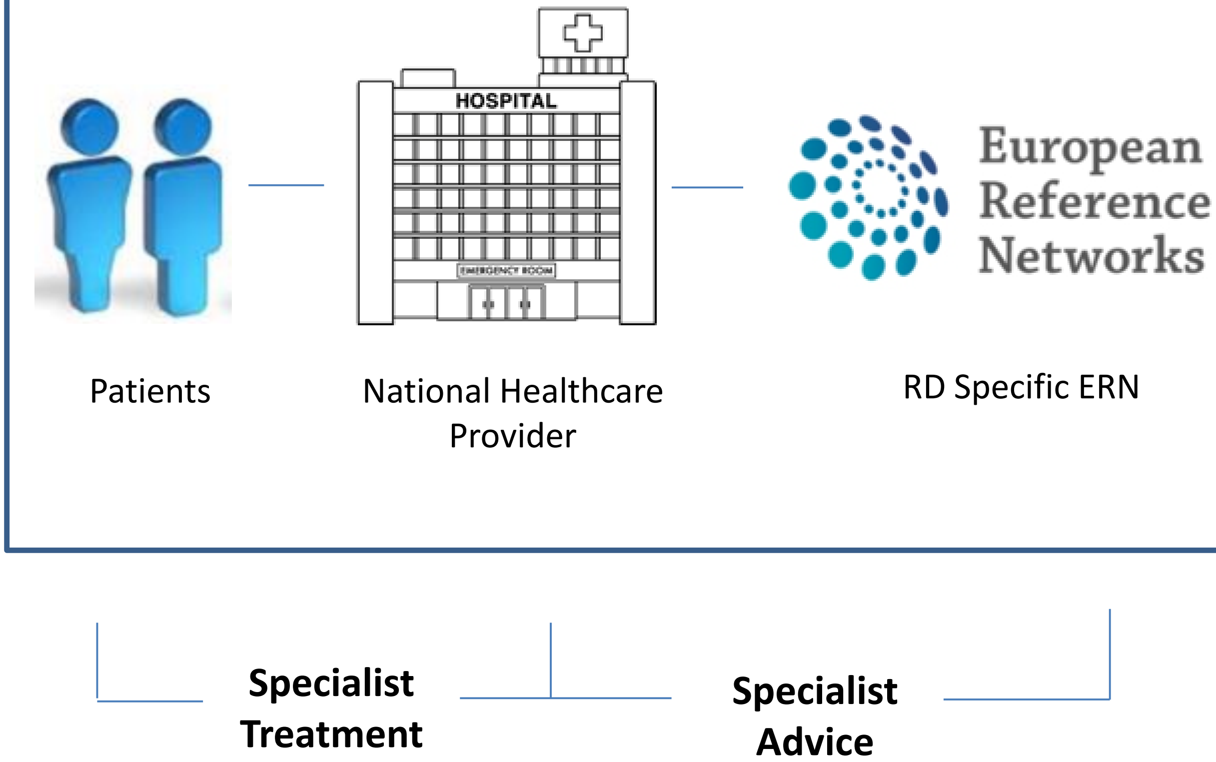
EURORDIS RARE DISEASES EUROPE

European Reference Network



Patients

National Healthcare Provider

RD Specific ERN

Specialist Treatment

Specialist Advice

- **Virtual healthcare**: specialist advice
- **Knowledge generation**: sharing experience and expertise, research and innovation
- **Knowledge dissemination**: clinical guidelines, healthcare pathways, education and training

Note: Slide inspired from Dr Enrique Terol, DG SANTE

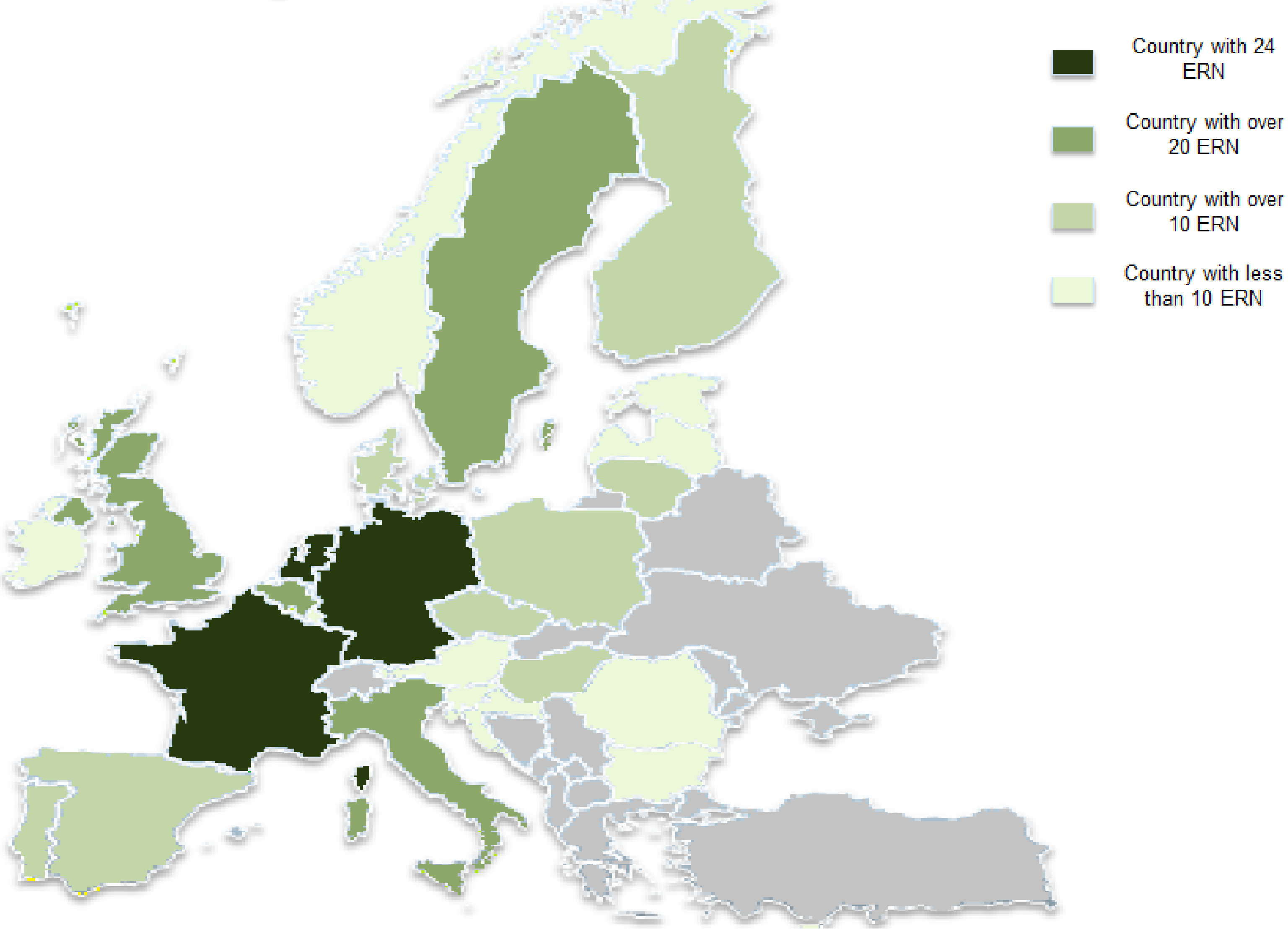
ERN Network Coordinators : Class of 2017

- Organising around these Thematic Groupings was a major undertaking
- Expected maybe 10-12 proposals in 2016: ended up with 24.
- All 24 ERNs are now officially approved:
  - Involved 370 hospitals and almost 1000 highly specialised units
  - 26 countries (25 EU MS + Norway) so far
  - Coordinating HCPs based in 7 countries

24 Approved ERNs : Our Optimal Structure

European Reference Networks	European Reference Networks
Rare Bone Diseases	Rare Immunodeficiency, Auto Inflammatory & Autoimmune Disease
Adult Cancer	Genetic Tumours
Peadatric Cancer	Rare Malformations & Development Anomalies
Rare Cardiac	Rare Hereditary Metabolic Diseases
Rare Connective Tissue	Rare Neurology
Rare Craniofacial & ENT	Rare Neuromuscular
Rare Endocrinology	Rare Pulmonary Diseases
Rare & Complex Epilepsy	Rare & Undiagnosed Skin Disorders
Rare Eye	Rare Renal
Rare Gastrointestinal Diseases	Transplantation in children
Rare Hematology	Rare Urogenital Diseases
Rare Hepatic Diseases	Rare Multi-systemic Vascular Diseases

ERN coverage in Member States



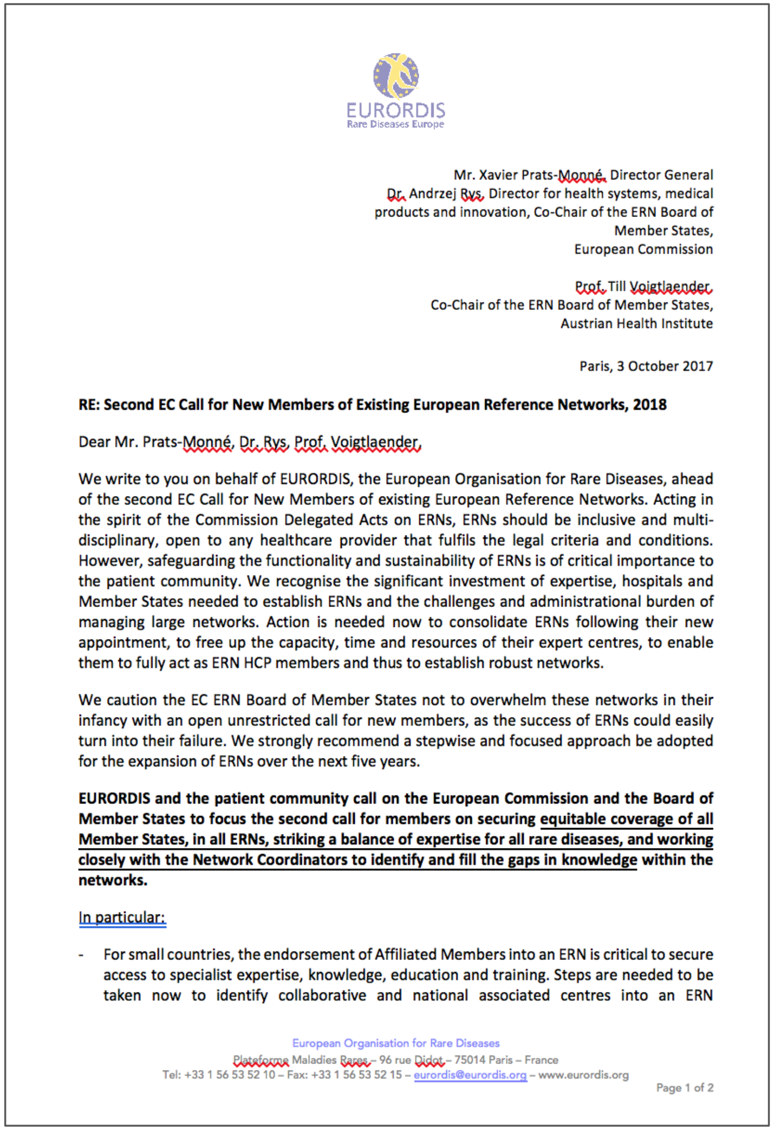
Governance for ERN Deployment

Board of Member States Working Groups	Network Coordinators Working Group
Monitoring WG	Monitoring WG
Information systems WG	Information systems WG
Affiliated Partners WG	Cross border and business continuity WG
Relationship with industry WG	Ethics WG
Integration with National healthcare systems WG	-
-	Research WG
-	Knowledge generation WG

→ **EURORDIS developing ePAG Transversal Working Groups:** For patient represents sitting on the their respective ERNs transversal working groups to come together and share experience and peer learning:

1. Research and Registries
2. eHealth and virtual healthcare
3. Education & Training
4. Quality, Outcomes & Guidelines

EURORDIS Advocacy



Mr. Xavier Prats-Monné, Director General
Dr. Andrzej Rys, Director for health systems, medical
products and innovation, Co-Chair of the ERN Board of
Member States,
European Commission

Prof. Till Voigtlaender,
Co-Chair of the ERN Board of Member States,
Austrian Health Institute

Paris, 3 October 2017

RE: Second EC Call for New Members of Existing European Reference Networks, 2018

Dear Mr. Prats-Monné, Dr. Rys, Prof. Voigtlaender,

We write to you on behalf of EURORDIS, the European Organisation for Rare Diseases, ahead of the second EC Call for New Members of existing European Reference Networks. Acting in the spirit of the Commission Delegated Acts on ERNs, ERNs should be inclusive and multi-disciplinary, open to any healthcare provider that fulfils the legal criteria and conditions. However, safeguarding the functionality and sustainability of ERNs is of critical importance to the patient community. We recognise the significant investment of expertise, hospitals and Member States needed to establish ERNs and the challenges and administrational burden of managing large networks. Action is needed now to consolidate ERNs following their new appointment, to free up the capacity, time and resources of their expert centres, to enable them to fully act as ERN HCP members and thus to establish robust networks.

We caution the EC ERN Board of Member States not to overwhelm these networks in their infancy with an open unrestricted call for new members, as the success of ERNs could easily turn into their failure. We strongly recommend a stepwise and focused approach be adopted for the expansion of ERNs over the next five years.

EURORDIS and the patient community call on the European Commission and the Board of Member States to focus the second call for members on securing equitable coverage of all Member States, in all ERNs, striking a balance of expertise for all rare diseases, and working closely with the Network Coordinators to identify and fill the gaps in knowledge within the networks.

In particular:

- For small countries, the endorsement of Affiliated Members into an ERN is critical to secure access to specialist expertise, knowledge, education and training. Steps are needed to be taken now to identify collaborative and national associated centres into an ERN

Advocacy Position on Call for New Members

- Need a stepwise and focused approach in the procedure of HCP's call
- An open unrestricted call for new members could easily turn into the failure of ERNs
- Need to work closely between MS and networks coordinators:
- To have an equitable coverage of all MS, in all expertise, in all ERN
- To take into account and respect the national networks on RD
- To continue assessment of HCP Members and refine criteria for expertise

Preparing for the Call for New HCP Members of Existing ERNs

Each network should:

- Update the **specific criteria for expertise**
- **Prepare a mapping document** assessing the geographical scientific and clinical gaps for each networks
- Define the networks development needs e.g.: types of competencies, number, territorial wish for full geographical coverage

HCP call : procedure divided in 3 steps

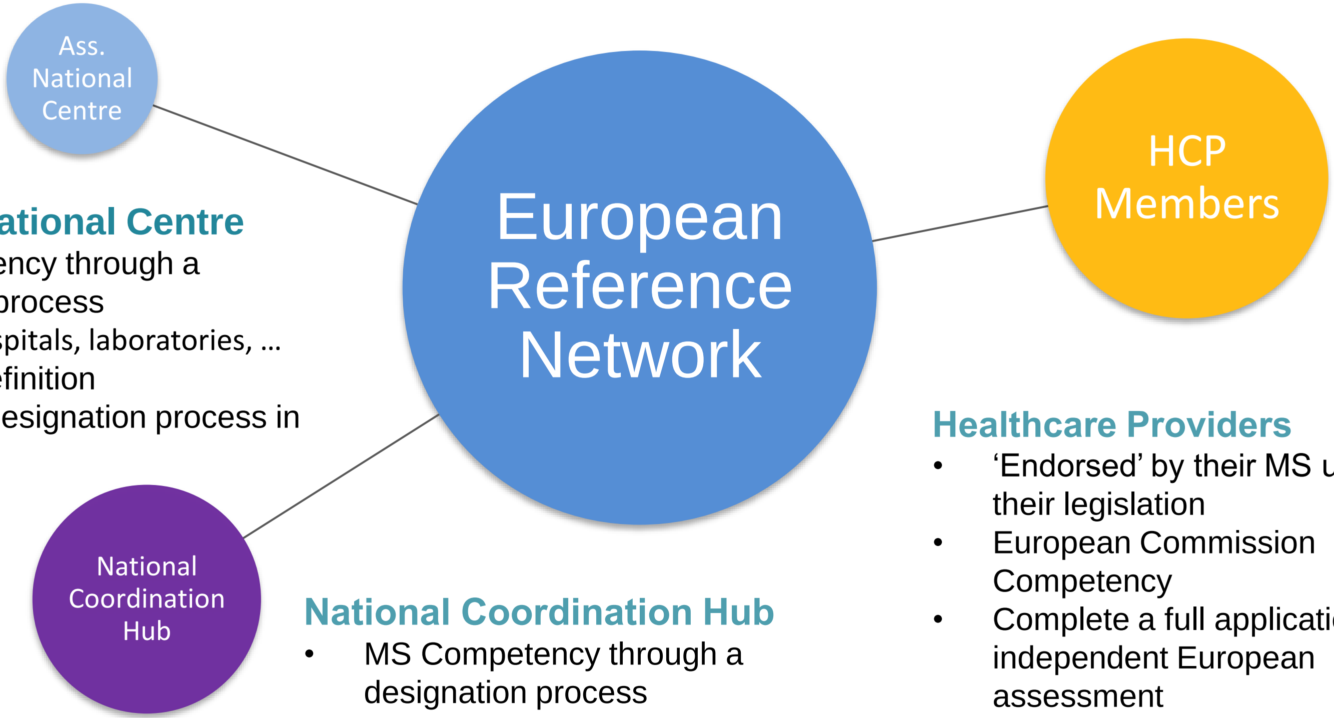
- Step 1: pre-assessment of application by each ERN (now to Dec 2017)
  - Production of an advisory opinion (not the end of the procedure if no favorable pre-assess as originally proposed by the board)
- Step 2: Call for new members of existing ERNs (Dec 2017 – March 2018)
  - Application developed and validated by member states
- Step 3: designation by EC according to three levels (April – June 2018)
  - Eligibility checking of the application by EC
  - Technical assessment by Independent Assessment Body
  - Final ERN BoMS approval (Summer 2018)

Note: estimated timescales, no official dates published

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.



Associated National Centre

- MS Competency through a designation process
- Clinicians, hospitals, laboratories, …
- No formal definition
- No defined designation process in MSs

Healthcare Providers

- 'Endorsed' by their MS under their legislation
- European Commission Competency
- Complete a full application and independent European assessment
- Role explicitly laid out in the EC ERN Delegated Acts

National Coordination Hub

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

Affiliated Partners: Draft position of the WG's board

1 priority

  - *"Priority for affiliated partners with no Full Member in a given ERN"*

- Reason for termination of the affiliation
  - "The case of the integration into an ERN of a Full Member from the MS of origins of the AP" in this case the new Full Member will represent the new focal point of the MS and the AF status is over

Process for designation

- ERNs Network Coordinators will receive a new version of the draft at the end of November
- As for the ERNs call, it is expected a position paper of the ERNs NC will be presented at the next BoMS Board in December
- Final decision concerning the prcedure of AP in December
- **No date agreed for the AP call**

Integration of ERNs into National Healthcare Systems

Some issues:

- How to harmonize legal frameworks on ERNs procedures?
- How to give legitimacy to ERNs : who gives the endorsement for an ERN?
- How is a patient referred to an ERN? (Does that GP have the capacity to refer to an ERN?)
- BoMS WG will be created in order to exchange on national practices and find some European solutions (4 network coordinators will be mandated on a voluntary basis)

Funding ERNs

Funding for coordination:

- **EC health programme grant** 23/24 ERNs each have 200k per year for 5 years.
- 60% DG SANTE funding +plus 40% co-funding by HCP Member's hospital trust and/or ministry (?)

Funding for infrastructure:

- **Registries grants** awarded x5 : Rare Metabolic ERN, Rare Endocrine ERN, xxx, xxx plus approval of the XXX but currently no funding available.

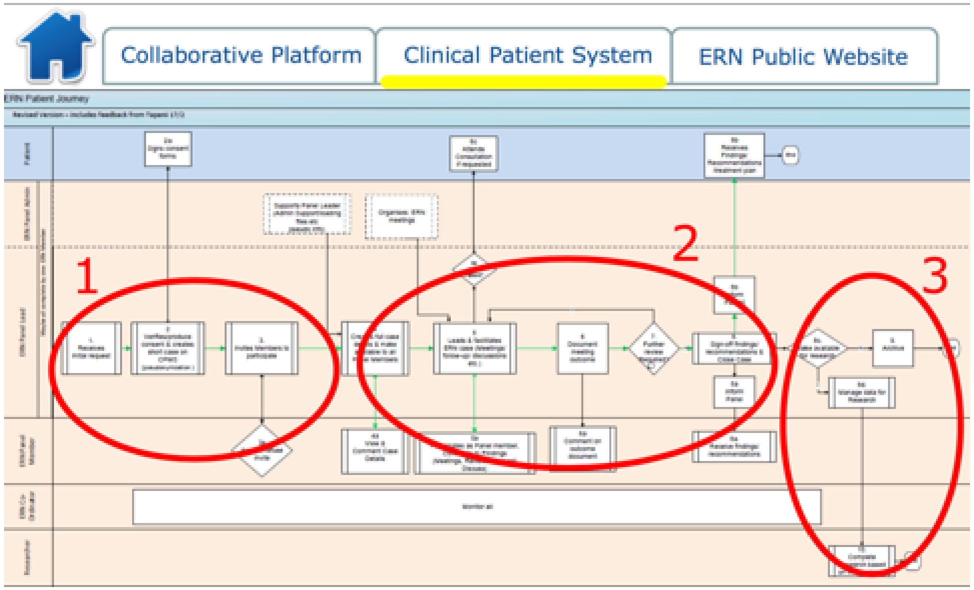
Funding for research:

- **Solving the Unsolved Proposal** which included Rare Neurology ERN, Rare Malformations & Intellectual Disabilities ERN, Genturis ERN, Rare Neuromuscular ERN
- **European Joint Programme** funding for ERN research activities

Data Infrastructure & Interoperability

ERN Clinical Patient Management System (in development):

- Data exchange/accessed for 1-to-1 care in theory to be stored/retained by the CPMS
- All data will be Pseudmonymised
- Potentially, hugely valuable repository of cases, for 'reuse'
- Manage data for research / archiving



Still questions to be answered:

- Link CPMS to registries in future?
- Commonality of data collection
- Exploring building interoperability:
  - Orphanet Nomenclature
  - FAIR Data
  - Human Phenotype Ontology
- Patient pathways yet to be confirmed
- When is a patient an ERN patient definition?

ERN Referral

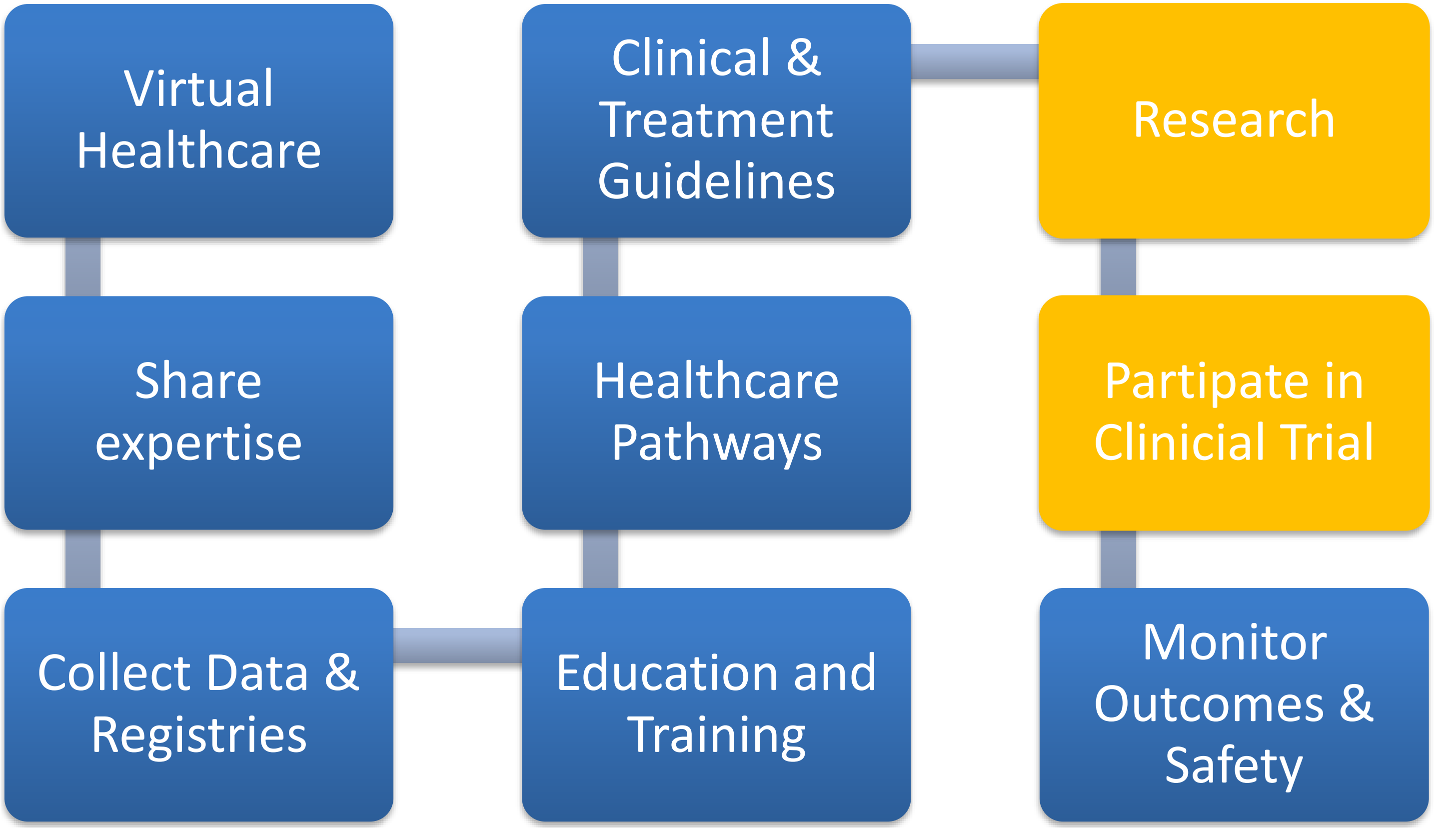
What is an ERN patient?

- *Patient cases that are reviewed by the ERN on the CPMS* vs *Total caseload of patients seen in an ERN HCP Member centre*?

How can a case be referred? No current formal referral process defined!

- Patient cases will be referred to the network via their national healthcare system and through the HCP Member of the Network (or Affiliated Partners).
- The Network is not directly accessible to individual patients
- However, patient cases can be referred to the relevant ERN HCP Member in their country by their local healthcare provider in cases where the patients' consent has been secured, in accordance with the rules of their national healthcare system

ERN Overview Activities



Virtual Healthcare

Share expertise

Collect Data & Registries

Education and Training

Healthcare Pathways

Clinical & Treatment Guidelines

Research

Partipate in Clinicial Trial

Monitor Outcomes & Safety

European Reference Networks Core Activities

Research
- Identify research gaps and carry out activities to fulfil these gaps
- Development of evidence base treatments
- Promote and support collborative research
- Coordinate access to data and samples
- Establish stronger linkages between research and industry

Clinical Trials
- Raise awareness of research projects and clinical trials
- Patient recruitment into clinical trials
- Assess feasibility of clinical trials
- Facilitate planning of clinical trials

Regulatory Procedures
- Orphan designation classification
- Scientific Advice Protocol Assistance
- Market Authorisation & Application Evaluation
- Post Market Authorisation

Healthcare
- Expert clinical opinion for diagnosis and treatment
- Transfer of knowledge on safe, effective and innovative medicine
- Clinical guidelines and treatment protocols
- Discuss new evidence-based treatments, therapies & technologies
- Real world experience of therapies

Research Activities

Clinical Trials

Market Authorisation

Healthcare delivery

Target Improvements in Rare Diseases

Information & data requirements:
- Production of evidence base and collect data & clinical information
- Support establishment of information networks, registries and databases
- Pool data to achieve sufficient sample size for epidemiology and clinical research
- Disseminate information on research projects and clinical trials in enable provider participation

- Pre and post data collection: real world post authorisation data

- Collects data on the use of medicines and medical technologies
- Clinical outcomes of treatment
- Share clinical information to support treatment

EURORDIS Roundtable of Companies

Draft Industry's 'wish-list' of ten high priority areas:

1. Early dialogue between stakeholders to define rules of engagement
2. Call for action to organise collaboration under a common goal between industry and ERN
3. ERNs need to be legal entities to enable contractual agreements
4. Access to ready-made community for research and therapeutic development
5. Research platform for open access data
6. Housing integrated validated disease based databases / registries
7. 'Real-life' laboratory for natural history studies
8. Transparency of outcomes across all therapeutic
9. Data collection over life cycle incl. post registration and real world data collection
10. *To be defined …*

Thank You