



**Update on ERN applications and endorsement of
Centres of Expertise by Member States,
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- Update on ERN applications & Technical Assessment
- Endorsement of Centres in ERNs
- ERNs & research infrastructure

Update on ERN applications and the technical assessment

Technical Assessment Process

Stage 3



Eligibility Check – 1st Part

- EC completed first part of eligibility check in **August 2016**.
- **All Networks successfully past**
- **<5 HCP not past due to lack of appropriate MS endorsement**

Eligibility Check – 2nd Part

- Independent Assessment Body completed second part of eligibility check at the **end September 2016**.

Stage 4



Technical Assessment - Documentation review

- ALL network applications reviewed
- SAMPLE number of HCPs including Network Coordinator.
- Completed **end October 2016**.

Technical Assessment - Onsite audits

- Coordinating Centre, plus one or two other HCP.
- Completed by the **mid-November 2016**.

Stage 5



Submitted technical assessment report

- IAB technical assessment report - for positive or negative assessment
- IAB submit ERN report to EC prior **15 November 2016**

Member States receive technical assessment reports on the 15 November 2016

Stage 6



Board of Member States Decision

- Approval of ERN application process and technical assessment on **14 December 2016**

EC announce successful ERNs in January 2017

EC ERN Third Conference to launch the successful ERNs, expected 8/9 March 2017

Multiple Technical Assessment Approach



Internal peer review – completed as part of the development of the network applications (stage 2)



External validation – MS endorsement as expert centre under MS legislation (stage 2)



External documentation review – first part of the technical assessment completed by Independent Assessment Body (stage 3)



Independent onsite audit - second part of the technical assessment (stage 4)

Evidence base

- Multiple methods of assessment is the strongest assessment of quality through the triangulation of information
- Targeted sampling to validate assessment in previous stage

Targeted Sampling

Peer Review (All)

All HCP have been peer reviewed by Network peers against *Specific Criteria* defining expertise and competency

All HCP endorsed by their MS, as an expert centre, under the MS legislation

Documentation Review (Sample)

All hospital have been independently review against *General Criteria* across all applications

370 Hospital provide, **960 HCP**, **24 ERN Network Applications**, in **26 Member State**

Sample of <10 HCP have a full **documentation review**, including the Co-ordinating Centre had a full documentation review

On-site Assessment (Sample of Sample)

Sample <4 HCP, including Coordinating Centre

ALL Member State have an On-site Assessment of one (or more) of their HCP
eurordis.org

Member State overview

Member State	No. ERNs / 25	Number HCP	Member State	No. ERNs / 24	Number HCP
Italy	23	192	Bulgaria	6	13
France	24 (ALL)	129	Romania	6	8
Germany	19	125	Slovenia	7	9
UK	23	118	Estonia	3	3
Netherlands	23	92	Croatia	2	2
Belgium	22	65	Austria	4	4
Spain	18	48	Norway	3	4
Czech Republic	19	29	Ireland	4	4
Sweden	22	31	Latvia	2	2
Portugal	16	29	Luxembourg	2	2
Poland	16	21	Malta	1	1
Denmark	12	17	Greece	0	0
Finland	13	15	Cyprus	0	0
Hungary	10	14	Slovak Republic	0	0
Lithuania	11	11	Total	-	989

Application overview

ERN Applications	No. HCP	ERN Applications	No. HCP
Rare Endocrinology	71	Rare Eye	30
Rare Heamatology	65	Rare Urogential Diseases	29
Rare Neurology	61	Rare Craniofacial & ENT	28
Rare Pulmonary Diseases	61	Rare Connective Tissue	26
Rare & Undiagnosed Skin Disorders	56	Rare & Complex Epilepsy	28
Peadatric Cancer	54	Rare Hepatic Diseases	28
Adult Cancer	67	Rare Cardiac	24
Rare Hereditary Metabolic Diseases	69	Rare Immunodeficiency, Auto Inflammatory & Autoimmune Diseases	24
Rare Neuromuscular	61	Genetic Tumours	23
Rare Malformations & Development Anomalies	37	Transplantation in children	20
Rare Bone Diseases	38	Rare Gastrointestinal Diseases	20
Rare Renal	38	<i>Rare Gynaecological & Obstetic Diseases</i>	0
Rare Multi-systemic Vascular Diseases	31		

Application Summary

- **Overlapping scope** across ERNs
- **Specific criteria and thresholds** need to be set for all rare diseases covered in an ERN
- **Holistic care** to be provided between ERN for multi-system RD, in multiple networks
- **'Twinning of network members'** between HCP with affiliated centres approach 😊
- **Adequate capacity** for HCP and Affiliated Partners to be active in a network
- **MS support and sustainable funding** to HCP and Affiliated Partners to support implementation

Quality Drivers

- Key drivers for quality that determine therapeutic results and survival of patients



Robust Information



**Timely referral,
diagnosis and
treatment**



**Core MDT team
presented in
diagnosis and
treatment planning)**



**Adoption and
adherence to
evidence based
protocols and best
practice guidelines**

ERN timeline

Time	Action
September – November 2016	Independent Assessment Body completes the technical assessment on ERN applications
16 November 2016	IAB submit positive assessment reports to EC & BoMS
15 December 2016	Board of Member States approval of successful ERN applications
January 2017	EC announce successful ERN applications
8, 9 March 2017	EC 3 rd ERC Conference, Lithuania – ERNs launched
March 2017 -2022	ERN's contracted for first 5 years
March 2017	Member States endorsement of Affiliated Partners, Collaborative Centres and National Centres as part of ERNs
2017 onwards	ERNs open application for new HCP members (EC expected to have an annual assessment for new HCP applications to approved ERNs)

Endorsement of Centres in ERNs

Overview

- European Reference Networks do not currently exist
- Accessibility and interoperability is key to the success of these networks.
- Connecting isolated experts and knowledge sharing
- Drive improvements in access and quality of healthcare ... within MS, not outside of MS
- Creating a common language for clinical dialogue based on quality data and outcomes
- Establish the 'common ground' of understanding of what ERNs will actual do.

EU added value = benefit within MS

Call for Action ... !

- EU add value = benefit within MS
- EU action = coordinated action at a National level in all MS !
- An EU action is a national opportunity ...

**European
Action**

Top-down approach:

- Anchor ERNs into national health systems

**National
Action**

Bottom-up approach:

- Build ERNs from a strong foundation in national health systems

Balanced Network Coverage

ERNs aim to be:

- Inclusive and foster a collaborative spirit
- Balanced and equal representation from its 'members' in all Member States.
- ERN Members to act as 'national gateway or hub' connecting the ERN to the national healthcare system, linking our local and regional hospitals and the ERN



ERN Members

	Healthcare Providers	Affiliated Partners
Identified	‘Endorsed’ by their MS under their legislation	‘Designated’ by their MS through a ‘transparent and explicit process’ in MS
Application Process	European Commission Competency: Complete a full application and independent European assessment against the EC ERN Delegated Acts by an independent assessment body	Member State Competency: Affiliate Partners do not complete the full application nor are assessed at a EU level
Role and function	HCP role and function is explicitly laid out in the EC ERN Delegated Acts	Definition is yet to be developed by the BoMS in 2017
Call	No information of future call for new ERNs. However, new hcp Members to an approved ERN will be annual.	MS will identify Affiliated Partners in 2017, ONLY for approved ERNs.

Three types of Affiliated Partners

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
- No definition
- No defined designation process in MSs

Collaborative National Centre

- MS Competency through a designation process
- No definition
- No defined designation process in MSs

Affiliated Partner

Healthcare Provider

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
- No definition
- No defined designation process in MSs

BoMS communication on National Centres, Affiliated and Collaborative Centres



Board of Member States

Statement adopted by the ERN BoMS on affiliated partners.

20 May 2016

The purpose of European Reference Networks (ERNs) is to enable the sharing of expertise and to improve access to care for patients across the European Union, especially for complex or rare medical conditions that require highly specialised healthcare and a concentration of knowledge and resources. In order for ERNs to deliver genuine added value to all European Union Member States, legislation makes provision for Member States which do not have representation from a member within an approved ERN to participate through **healthcare providers that are designated by their Member State** as “associated” and or “collaborative” national centres. Member States may also wish to designate a national coordination hub with all types of Networks.

The ERN Board of Member States fully supports the objectives set out in the Commission Implementing Decision (2014/287/EU) and as well as to facilitate the participation in ERNs of as many as possible Member States, none of whose healthcare providers are Members of an approved Network as indicated in the Preamble (14) of the Commission Delegated Decision (2014/286/EU), in order to achieve the widest possible geographical coverage, exchange of knowledge and best practice.

Healthcare providers from those Member States might be designated as:

- a. “Associated National Centres” focusing in the provision of healthcare,
- b. “Collaborative National Centres” focusing on the production of knowledge and tools to improve the quality of care.

Strategy for success

Integration of ERNs with national healthcare systems:



Multiple Technical Assessment Approach

1

- Creation of National Networks
- Connect national 'nodes' centres between ERNs and regional hospitals
- Identification of Affiliated Centres in 2017
- Promote new HCP applications in approved ERHs
- Raise awareness and participation of regional hospitals in ERN education, training
- Active involvement and monitor the HCP actions plans outlined in ERN applications

2

- Anchor ERN evidence-base pathways and adoption of ERN evidence base practice in locally
- Review HCP self-assessment and action plans, and HCP Operational
- Monitor quality and national expertise against ERN specific criteria and thresholds for expertise by disease area.
- Use ERN best-practice protocols to drive access to a increased quality and basket of care
- Sign posting patients into ERN's HCP and supporting patients rights for prior approval

3

- Identify national needs for rare diseases through adoption of common codification
- Understand burden of disease and spend on rare disease and concentration of spend to RD
- Improve quality of databases, registries to enable thereuptic drug development for conditions a without a treatment + research into undiagnosed populations

European Reference Networks & Research Infrastructure

ERNs & Research Infrastructure

**European
Reference
Networks**

**Research
Networks**

**Research
Infrastructure**

Data sharing is the key to unlock the potential of ERNs:

- Creates a common language for clinicians to share expertise and identify new innovation and emerging best practice.
- Connecting ERNs with core research infrastructure, e.g.: ERCT with Rare Cancer ERNs as a common research network; connecting RD ERNs with Undiagnosed Disease Research Networks



INFRAFRONTIER
mouse disease models



EATRIS
European Infrastructure for
Translational Medicine



BBMRI-ERIC®
Biobanking and
BioMolecular resources
Research Infrastructure



ECRIN
EUROPEAN CLINICAL RESEARCH INFRASTRUCTURES NETWORK



**European
Reference
Networks**



innovative
medicines
initiative



WEB-RADR



ADAPTSMART

RD  **Connect**



**Rare
Best
Practices**



**GENETICS
CLINIC OF THE FUTURE**



EUPATI
European Patients' Academy
on Therapeutic Innovation



EURORDIS
Rare Diseases Europe

European Research Infrastructures.

1. **BBMRI**—the Biobanking and Biomolecular Resources Research Infrastructure. (<http://bbmrierc.eu/>).
2. **EATRIS**—The research infrastructure for translational medicine. (<http://www.eatris.eu>).
3. **ECRIN**—The European Clinical Research Infrastructure Network. (<http://www.ecrin.org>).
4. **ELIXIR**—the pan-European research infrastructure for biological information. (<http://www.elixireurope.org>).
5. **Infrafrontier**—The infrastructure for mouse disease models and phenotype data. (<http://www.infrafrontier.eu>).
6. **Instruct**—integrated structural biology unlocking the secrets of life. (<http://www.structuralbiology.eu>).
7. **EU-OPENSREEN**—the European Infrastructure of Open Screening Platforms for Chemical Biology. (<http://www.eu-openscreen.de>).
8. **EMBRC**—the European Marine Biological Resource Centre. (<http://www.embrc.eu>).
9. **Euro-BioImaging**—the research infrastructure for imaging technologies, (<http://www.eurobioimaging.eu>).
10. **ISBE**—the Infrastructure for Systems Biology in Europe. (<http://project.isbe.eu>).
11. **MIRRI**—the microbial resource research infrastructure. (<http://www.mirri.org>).

Key Message

- *TO BE COMPLETED*

Thank you



Daniel -Sanfilippo syndrome