Leids Universitair
Medisch Centrum

Building the European Reference Network for Rare Endocrine Conditions (Endo-ERN)

Journee Annuelle, FIRENDO, Paris, 14 december 2016

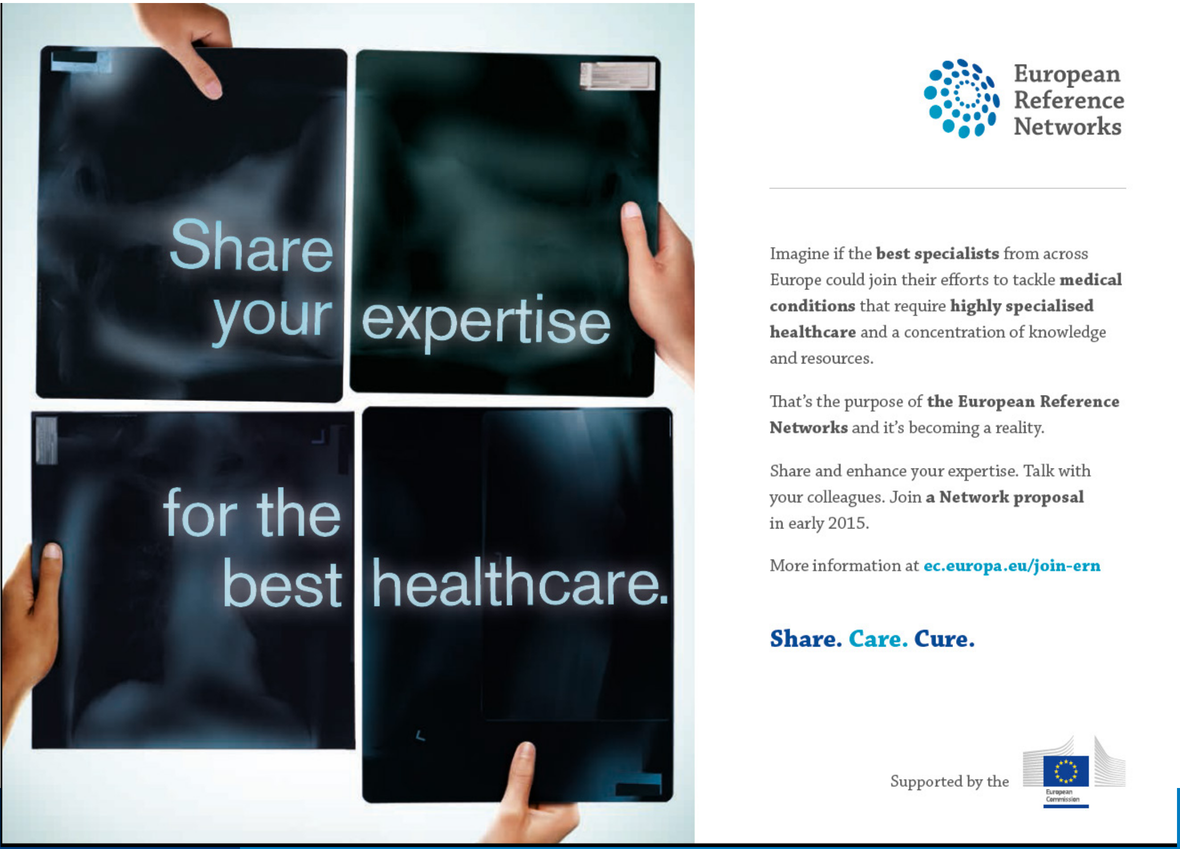
European Reference Networks: Scope and Context

Article 12 *Directive of patients' rights in cross-border healthcare*

Networks of healthcare providers aiming at:

Improving quality and safety and access to highly specialised healthcare across the EU

Patients affected by rare or low prevalence and complex diseases



Imagine if the **best specialists** from across Europe could join their efforts to tackle **medical conditions** that require **highly specialised healthcare** and a concentration of knowledge and resources.

That's the purpose of **the European Reference Networks** and it's becoming a reality.

Share and enhance your expertise. Talk with your colleagues. Join **a Network proposal** in early 2015.

More information at **ec.europa.eu/join-ern**

Share. Care. Cure.

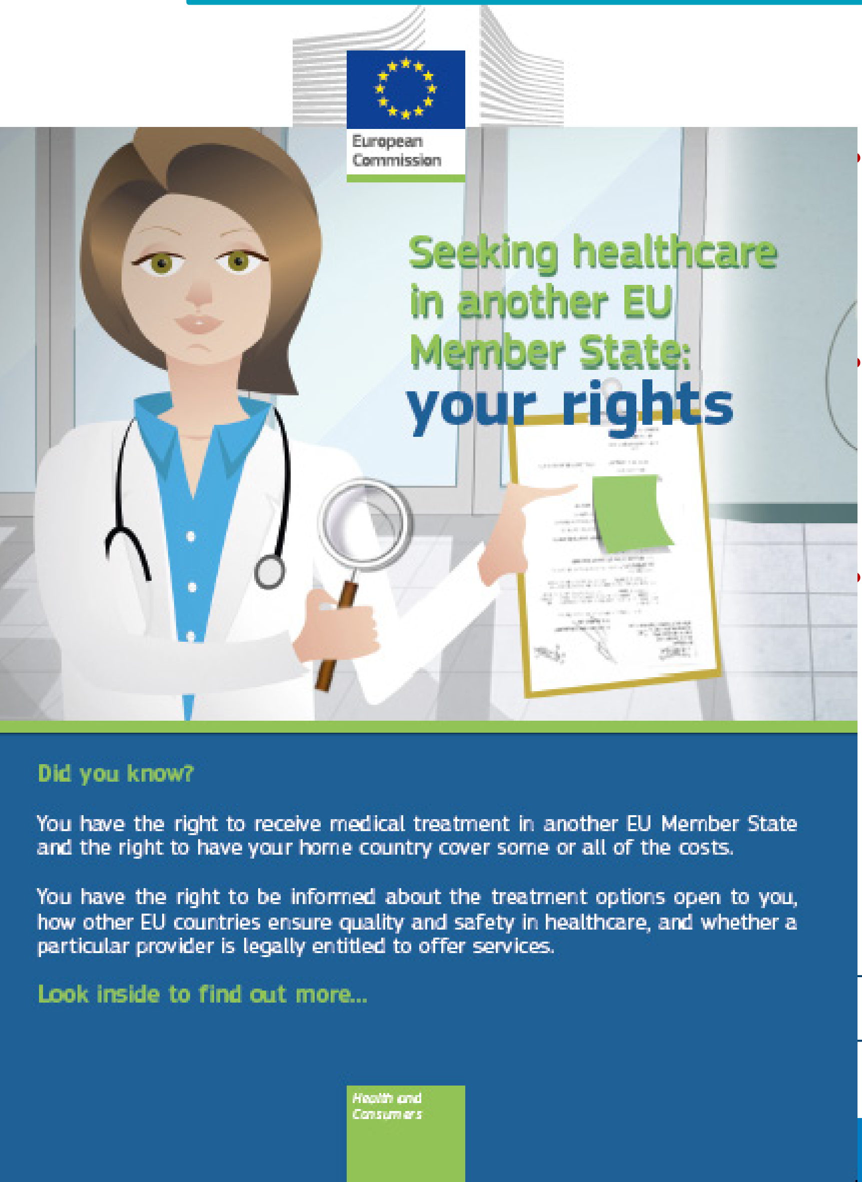
Added value (EU level)

Need for Cooperation:

- ✓ Scarcity of knowledge
- ✓ Need for education
- ✓ Complexity / high costs
- ✓ Effective use of resources

Key issues adressed by the directive

Directive 2011/24/EU of patients' rights in cross-border healthcare



European Commission

Seeking healthcare in another EU Member State: your rights

Did you know?

You have the right to receive medical treatment in another EU Member State and the right to have your home country cover some or all of the costs.

You have the right to be informed about the treatment options open to you, how other EU countries ensure quality and safety in healthcare, and whether a particular provider is legally entitled to offer services.

Look inside to find out more...

Health and Consumers

- Right to choose and be reimbursed for healthcare provided by public or private providers located in the EU
- More transparency about their rights, treatment options or, the quality and safety levels of healthcare providers
- Strong focus on cooperation among Member States
  - ○ Mutual recognition of prescription
  - ○ eHealth
  - ○ Health Technology Assessment
  - ○ **European Reference Networks**

Entry into force at National level 25 October 2013

National actions at the level of the Board of Member States:

Ministeries of Health:
National evaluation of expertise centres for rare diseases

Recognition of expertise centres for rare endocrine diseases: national endorsement

October 2014 - jan 2016

The trajectory towards an European Reference Network for Rare Endocrine Conditions

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Lisbon 7-8 oct
2nd ERN conference

Olaf Hiort (ESPE)

Jérôme Bertherat (ESE)

Alberto Pereira

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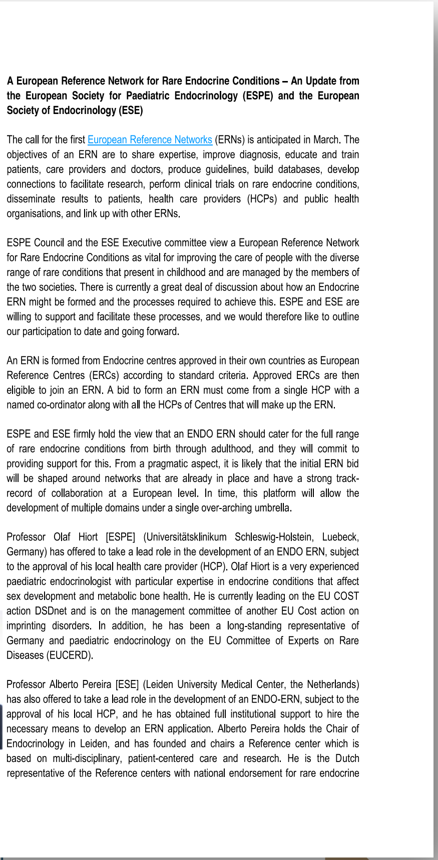
1st ESPE-ESE statement



A European Reference Network for Rare Endocrine Conditions – An Update from the European Society for Paediatric Endocrinology (ESPE) and the European Society of Endocrinology (ESE)

The call for the first European Reference Networks (ERNs) is anticipated in March. The objectives of an ERN are to share expertise, improve diagnosis, educate and train patients, care providers and doctors, produce guidelines, build databases, develop connections to facilitate research, perform clinical trials on rare endocrine conditions, disseminate results to patients, health care providers (HCPs) and public health organisations, and link up with other ERNs.

ESPE Council and the ESE Executive committee view a European Reference Network for Rare Endocrine Conditions as vital for improving the care of people with the diverse range of rare conditions that present in childhood and are managed by the members of the two societies. There is currently a great deal of discussion about how an Endocrine ERN might be formed and the processes required to achieve this. ESPE and ESE are willing to support and facilitate these processes, and we would therefore like to outline our participation to date and going forward.

An ERN is formed from Endocrine centres approved in their own countries as European Reference Centres (ERCs) according to standard criteria. Approved ERCs are then eligible to join an ERN. A bid to form an ERN must come from a single HCP with a named co-ordinator along with all the HCPs of Centres that will make up the ERN.

ESPE and ESE firmly hold the view that an ENDO ERN should cater for the full range of rare endocrine conditions from birth through adulthood, and they will commit to providing support for this. From a pragmatic aspect, it is likely that the initial ERN bid will be shaped around networks that are already in place and have a strong track-record of collaboration at a European level. In time, this platform will allow the development of multiple domains under a single over-arching umbrella.

Professor Olaf Hiort [ESPE] (Universitätsklinikum Schleswig-Holstein, Luebeck, Germany) has offered to take a lead role in the development of an ENDO ERN, subject to the approval of his local health care provider (HCP). Olaf Hiort is a very experienced paediatric endocrinologist with particular expertise in endocrine conditions that affect sex development and metabolic bone health. He is currently leading on the EU COST action DSDnet and is on the management committee of another EU Cost action on imprinting disorders. In addition, he has been a long-standing representative of Germany and paediatric endocrinology on the EU Committee of Experts on Rare Diseases (EUCERD).

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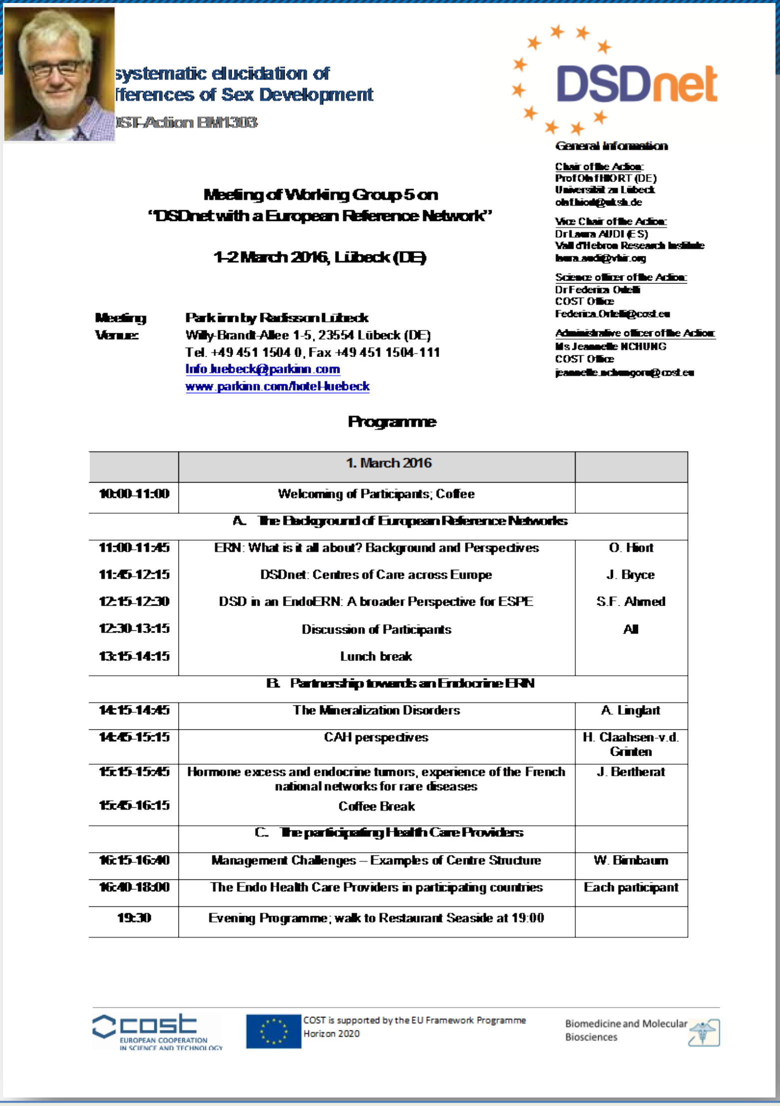


systematic elucidation of
fferences of Sex Development
ST-Action BM1303

General Information

Chair of the Action:
Prof Olaf HIORT (DE)
Universität zu Lübeck
olaf.hiort@uksh.de

Vice Chair of the Action:
Dr Laura AUDI (ES)
Vall d'Hebron Research Institute
laura.audi@vhir.org

Science officer of the Action:
Dr Federica Ortelli
COST Office
Federica.Ortelli@cost.eu

Administrative officer of the Action:
Ms Jeannette NCHUNG
COST Office
jeannette.nchungoru@cost.eu

Meeting of Working Group 5 on
"DSDnet with a European Reference Network"

1-2 March 2016, Lübeck (DE)

Meeting Venue: Park inn by Radisson Lübeck
Willy-Brandt-Allee 1-5, 23554 Lübeck (DE)
Tel. +49 451 1504 0, Fax +49 451 1504-111
Info.luebeck@parkinn.com
www.parkinn.com/hotel-luebeck

Programme

	1. March 2016	
10:00-11:00	Welcoming of Participants; Coffee	
	A. The Background of European Reference Networks	
11:00-11:45	ERN: What is it all about? Background and Perspectives	O. Hiort
11:45-12:15	DSDnet: Centres of Care across Europe	J. Bryce
12:15-12:30	DSD in an EndoERN: A broader Perspective for ESPE	S.F. Ahmed
12:30-13:15	Discussion of Participants	All
13:15-14:15	Lunch break	
	B. Partnership towards an Endocrine ERN	
14:15-14:45	The Mineralization Disorders	A. Linglart
14:45-15:15	CAH perspectives	H. Claahsen-v.d. Grinten
15:15-15:45	Hormone excess and endocrine tumors, experience of the French national networks for rare diseases	J. Bertherat
15:45-16:15	Coffee Break	
	C. The participating Health Care Providers	
16:15-16:40	Management Challenges – Examples of Centre Structure	W. Birnbaum
16:40-18:00	The Endo Health Care Providers in participating countries	Each participant
19:30	Evening Programme; walk to Restaurant Seaside at 19:00	

COST is supported by the EU Framework Programme Horizon 2020

Biomedicine and Molecular Biosciences

Rare Endocrine Diseases ERN

NEWSLETTER Update

March 21, 2016

Symposium 17 March 2016

Dear Colleagues,

The Leiden Rare Endocrine Diseases ERN Symposium was held on Thursday 17 March 2016. The purpose of the meeting was first of all to inform and update all those interested in joining an Endo ERN on the goals and the trajectory towards a broad thematic Endo ERN, in view of the perspectives of ESE and ESPE. Furthermore, the symposium aimed to provide a platform to discuss how to further develop and shape the ERN within the provided framework of the call, but importantly, also according to the wishes and needs that had emerged during the past trajectory. Sixty persons (both paediatric and adult endocrinologists) representing 14 different EU countries attended the meeting.

Welcome by the Dean, prof. Hogendoorn

The plenaries in the morning exemplified that the development and shaping of the ERN is a 'bottom-up' procedure. We anticipate a broad thematic, and very successful ERN with a common denominator that unifies both pediatric and adult endocrinologists: 'excellency in diagnostics and treatment of hormone disturbances throughout life'. As such, there was a broad consensus that all those that provide unique expertise in a specific area of rare endocrine diseases should be offered a place in the 'overarching' network, provided that they fulfill the operational criteria for HCPs and have obtained national endorsement.

With respect to the structure of the ERN 8 subthemes were defined representing 8 subnetworks: Adrenal, DSD, Growth Disorders, Pituitary, Thyroid, Men & other genetic syndromes, Lipodystrophy/syndrome obesity, and possibly genetic diabetes and Rare Parathyroid Disorders.

In order to keep such a large ERN manageable, it is necessary to adjust the structure and governance of the board to these specific needs. This could be ensured by a multi-layer board, for instance composed of 1) an ERN management team (Chair + vice-chair (adult and

1 march 2016 Lubeck

17 march 2016 Leiden

The trajectory towards an European Reference Network for Rare Endocrine Conditions

EndoERN: broad thematic network ensuring all patients have 'a home'

excellency in diagnostics and treatment of hormonal disturbances throughout life

ESPE
European Society for Paediatric Endocrinology

European Society of Endocrinology
the European hormone society

Adrenal - Genetic Diabetes – Growth – DSD – Genetic Endocrine Syndromes – Pituitary - Thyroid

→

birth senescence

The development of the European landscape for Rare Endocrine Conditions


Workshop men- and other genetic endocrine syndromes


Workshop Pituitary

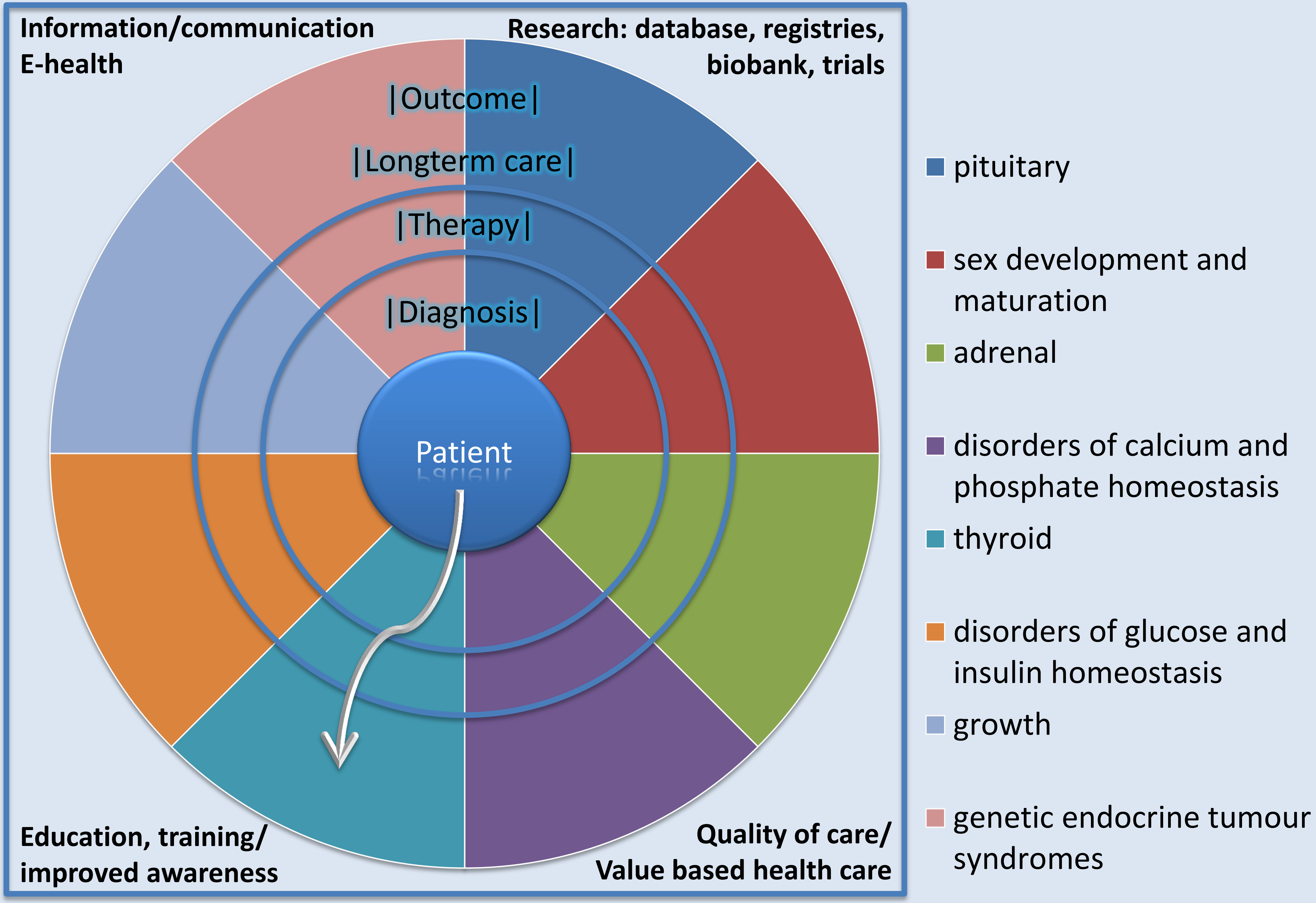

Workshop Growth disorders


Workshop Adrenal


Workshop Thyroid


Workshop DSD

Rare Endocrine Conditions ERN: Main Thematic grouping



Information/communication
E-health

Research: database, registries,
biobank, trials

|Outcome|

|Longterm care|

|Therapy|

|Diagnosis|

Patient

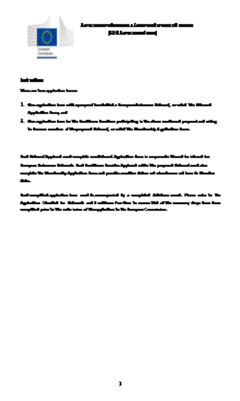
Education, training/
improved awareness

Quality of care/
Value based health care

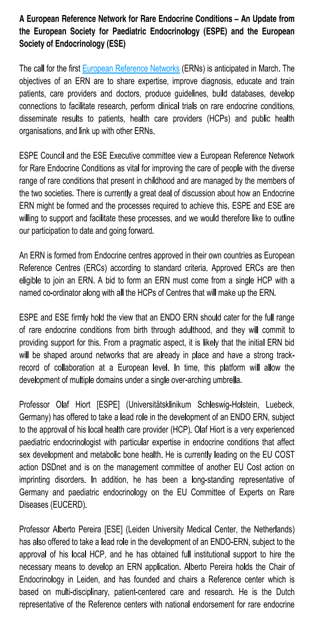
- ■ pituitary
- ■ sex development and maturation
- ■ adrenal
- ■ disorders of calcium and phosphate homeostasis
- ■ thyroid
- ■ disorders of glucose and insulin homeostasis
- ■ growth
- ■ genetic endocrine tumour syndromes

2nd ESPE-ESE statement
(with main thematic groupings)

ERN call



Boston meeting:
ESE-ESPE Decision on Endo-ERN coordination



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16 march 16

2 april 2016

7 april 2016

ESPE
European Society for
Paediatric Endocrinology

The Network Application

European Society
of Endocrinology
the European hormone society

7/4 Joint newsletter from endocrine societies

- action ESE/ESPE

30/4 install writing committee

- Report 7/5, 14/5, 28/5, 3/6, 10/6, 17/6

Invite all HCP to Sharepoint endo ERN website

- action Leiden Task Force

14/5 have determined thematic HCP criteria

- action Chairs

14/5 Start grant application for funding

- action writing committee

ECE 2016 Munich: Sunday 29 may 2016
Endo-ERN writing committee meeting

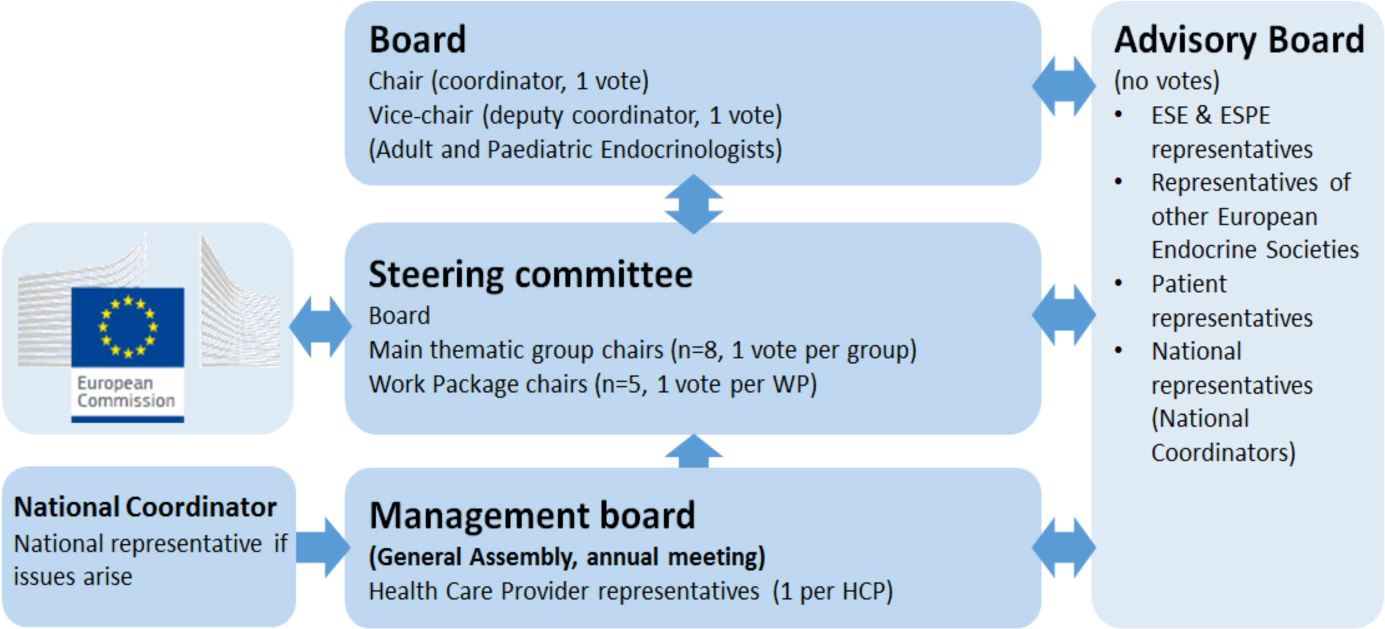
6/6 Ultimate deadline HCP application to network

21/6 Ultimate deadline ERN network application to EU Commission

The Network (Application)

European
Reference
Networks

Governance Rare Endocrine Conditions ERN (Endo-ERN)



Board

Chair (coordinator, 1 vote)
Vice-chair (deputy coordinator, 1 vote)
(Adult and Paediatric Endocrinologists)

Steering committee

Board
Main thematic group chairs (n=8, 1 vote per group)
Work Package chairs (n=5, 1 vote per WP)

National Coordinator

National representative if issues arise

Management board

(General Assembly, annual meeting)

Health Care Provider representatives (1 per HCP)

Advisory Board

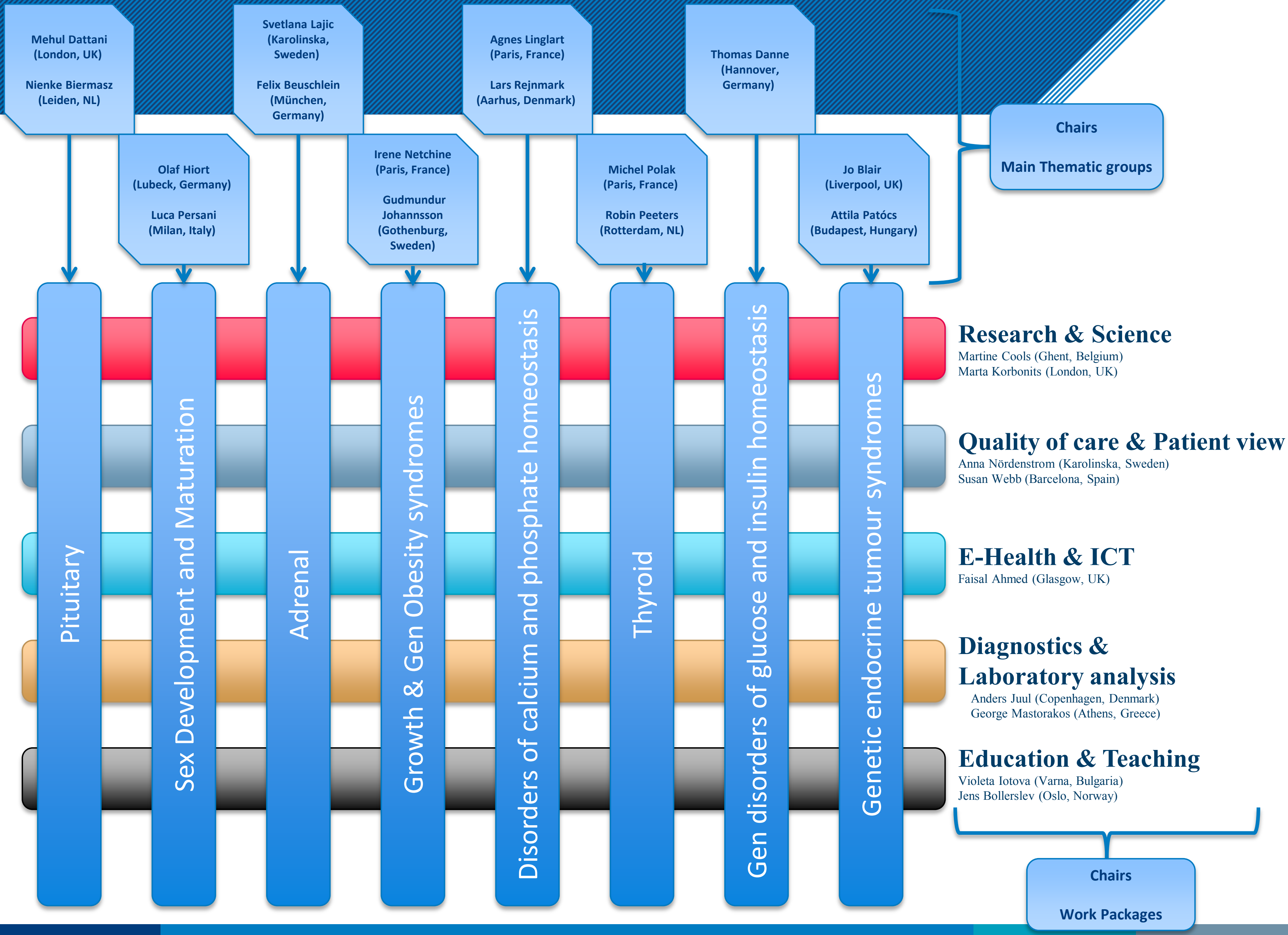
(no votes)

- ESE & ESPE representatives
- Representatives of other European Endocrine Societies
- Patient representatives
- National representatives (National Coordinators)

European Society of Endocrinology
the European hormone society

ESPE
European Society for Paediatric Endocrinology

European Reference Networks



Chairs

Main Thematic groups

Pituitary
Mehul Dattani (London, UK)
Nienke Biermasz (Leiden, NL)

Sex Development and Maturation
Olaf Hiort (Lubeck, Germany)
Luca Persani (Milan, Italy)

Adrenal
Svetlana Lajic (Karolinska, Sweden)
Felix Beuschlein (München, Germany)

Growth & Gen Obesity syndromes
Irene Netchine (Paris, France)
Gudmundur Johannsson (Gothenburg, Sweden)

Disorders of calcium and phosphate homeostasis
Agnes Linglart (Paris, France)
Lars Rejnmark (Aarhus, Denmark)

Thyroid
Michel Polak (Paris, France)
Robin Peeters (Rotterdam, NL)

Gen disorders of glucose and insulin homeostasis
Thomas Danne (Hannover, Germany)

Genetic endocrine tumour syndromes
Jo Blair (Liverpool, UK)
Attila Patócs (Budapest, Hungary)

Chairs

Work Packages

Research & Science
Martine Cools (Ghent, Belgium)
Marta Korbonits (London, UK)

Quality of care & Patient view
Anna Nördenstrom (Karolinska, Sweden)
Susan Webb (Barcelona, Spain)

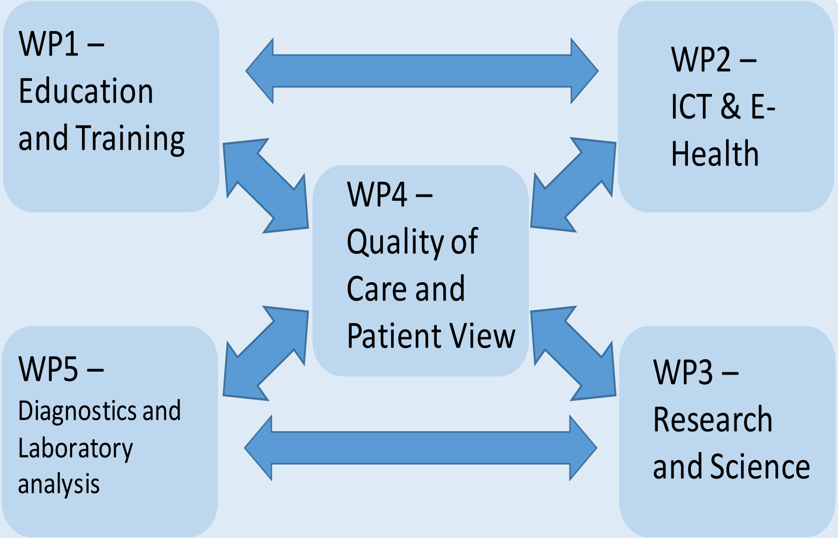
E-Health & ICT
Faisal Ahmed (Glasgow, UK)

Diagnostics & Laboratory analysis
Anders Juul (Copenhagen, Denmark)
George Mastorakos (Athens, Greece)

Education & Teaching
Violeta Iotova (Varna, Bulgaria)
Jens Bollerslev (Oslo, Norway)

The mission of Endo-ERN

Endo-ERN's mission is to reduce and ultimately abolish inequalities in care for patients with rare endocrine disorders in Europe, through facilitating knowledge sharing and facilitating related healthcare and research. Endo-ERN provides equality between paediatric and adult patients. Ultimately, Endo-ERN will result in the best possible care for every patient with a rare endocrine condition.


WP1 – Education and Training

WP2 – ICT & E-Health

WP4 – Quality of Care and Patient View

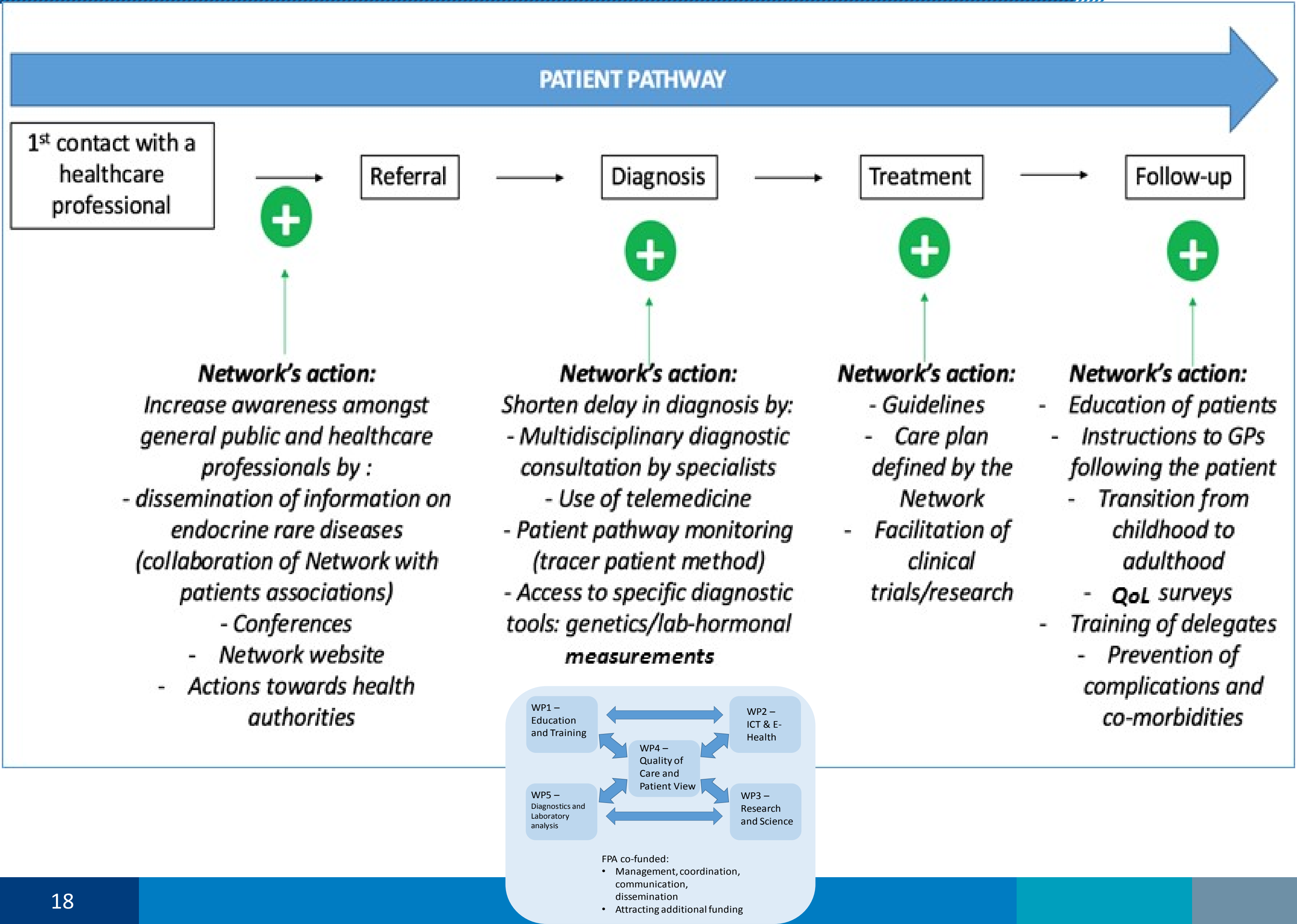
WP5 – Diagnostics and Laboratory analysis

WP3 – Research and Science

FPA co-funded:

- Management, coordination, communication, dissemination
- Attracting additional funding

Endo-ERN Work program structure



PATIENT PATHWAY

1st contact with a healthcare professional → Referral → Diagnosis → Treatment → Follow-up

Network's action:
Increase awareness amongst general public and healthcare professionals by :
- dissemination of information on endocrine rare diseases (collaboration of Network with patients associations)
- Conferences
- Network website
- Actions towards health authorities

Network's action:
Shorten delay in diagnosis by:
- Multidisciplinary diagnostic consultation by specialists
- Use of telemedicine
- Patient pathway monitoring (tracer patient method)
- Access to specific diagnostic tools: genetics/lab-hormonal ***measurements***

Network's action:
- Guidelines
- Care plan defined by the Network
- Facilitation of clinical trials/research

Network's action:
- Education of patients
- Instructions to GPs following the patient
- Transition from childhood to adulthood
- ***QoL*** *surveys*
- Training of delegates
- Prevention of complications and co-morbidities

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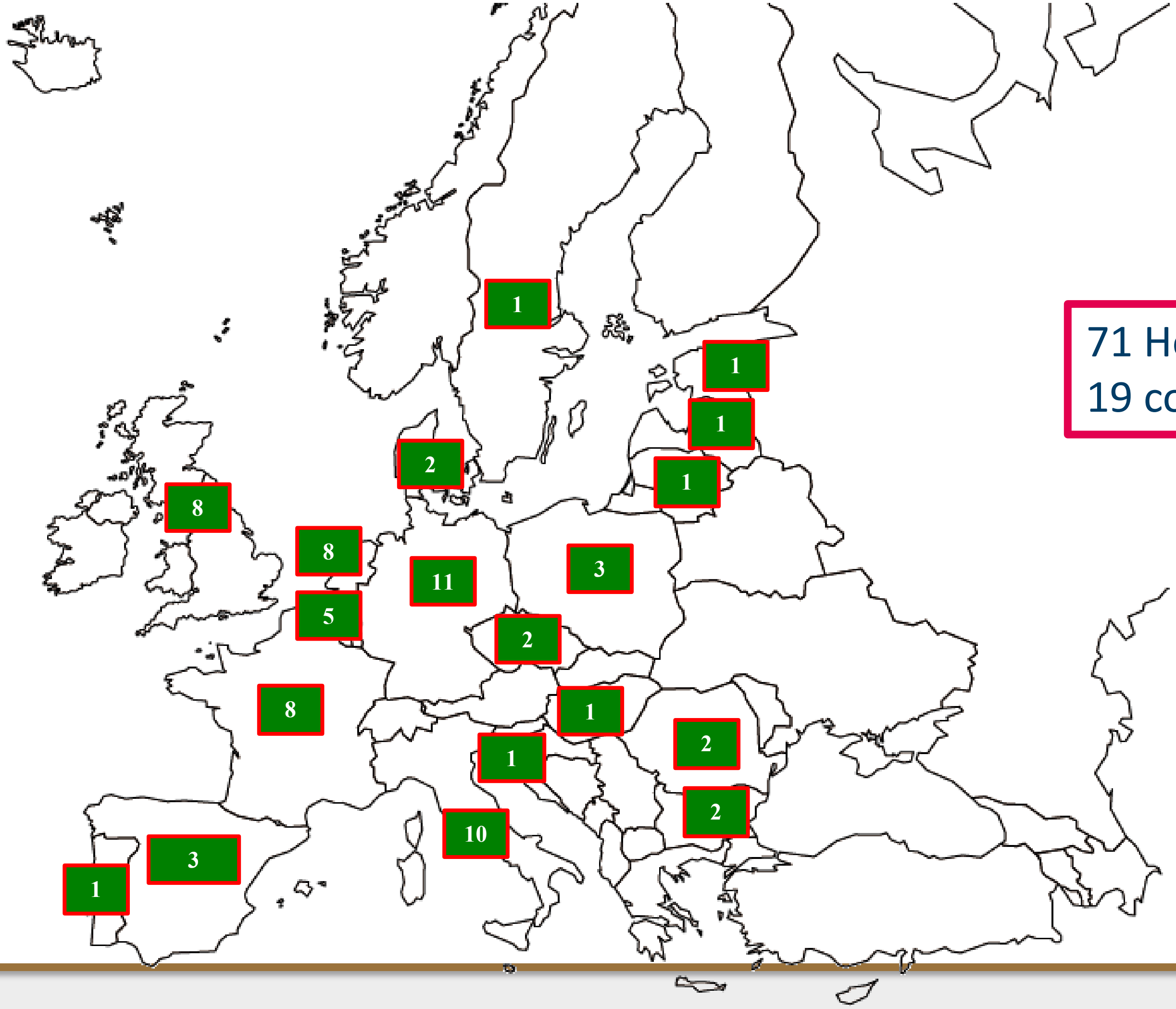
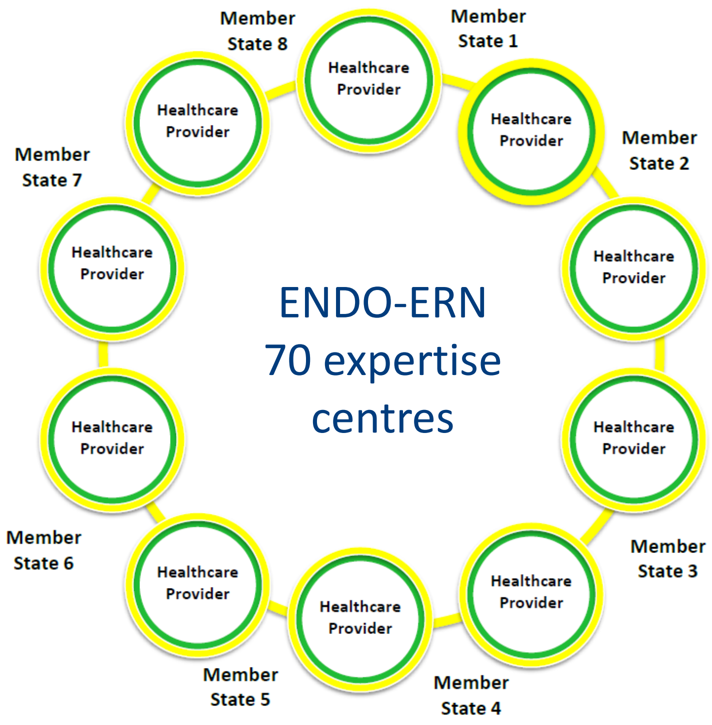
Endo-ERN: Distribution of HCPs by country



71 Health Care Providers
19 countries

European network of Expertise Centers: how can it work optimally?



ENDO-ERN
70 expertise centres

Shorten delay in diagnosis:
- Earlier referral
- All required experts together
- Best diagnostic techniques

Best treatment
Best education of all involved

European Reference Networks

European Network of expertise centers: patient travels to expertise?

patient with rare endocrine condition

patient with rare endocrine condition

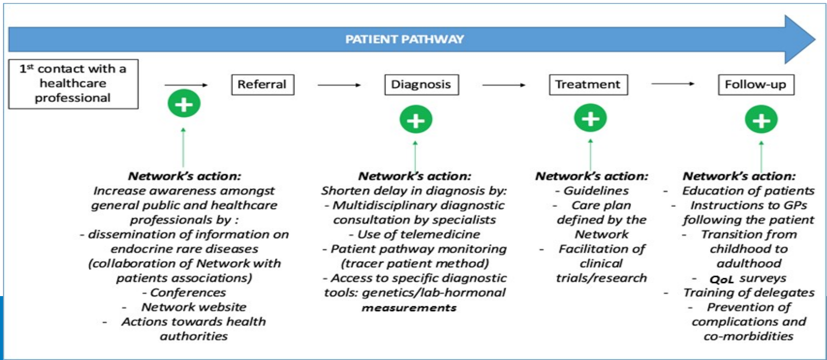
patient with rare endocrine condition

patient with rare endocrine condition

patient with rare endocrine condition

patient with rare endocrine condition

ENDO-ERN
70 expertise
centres



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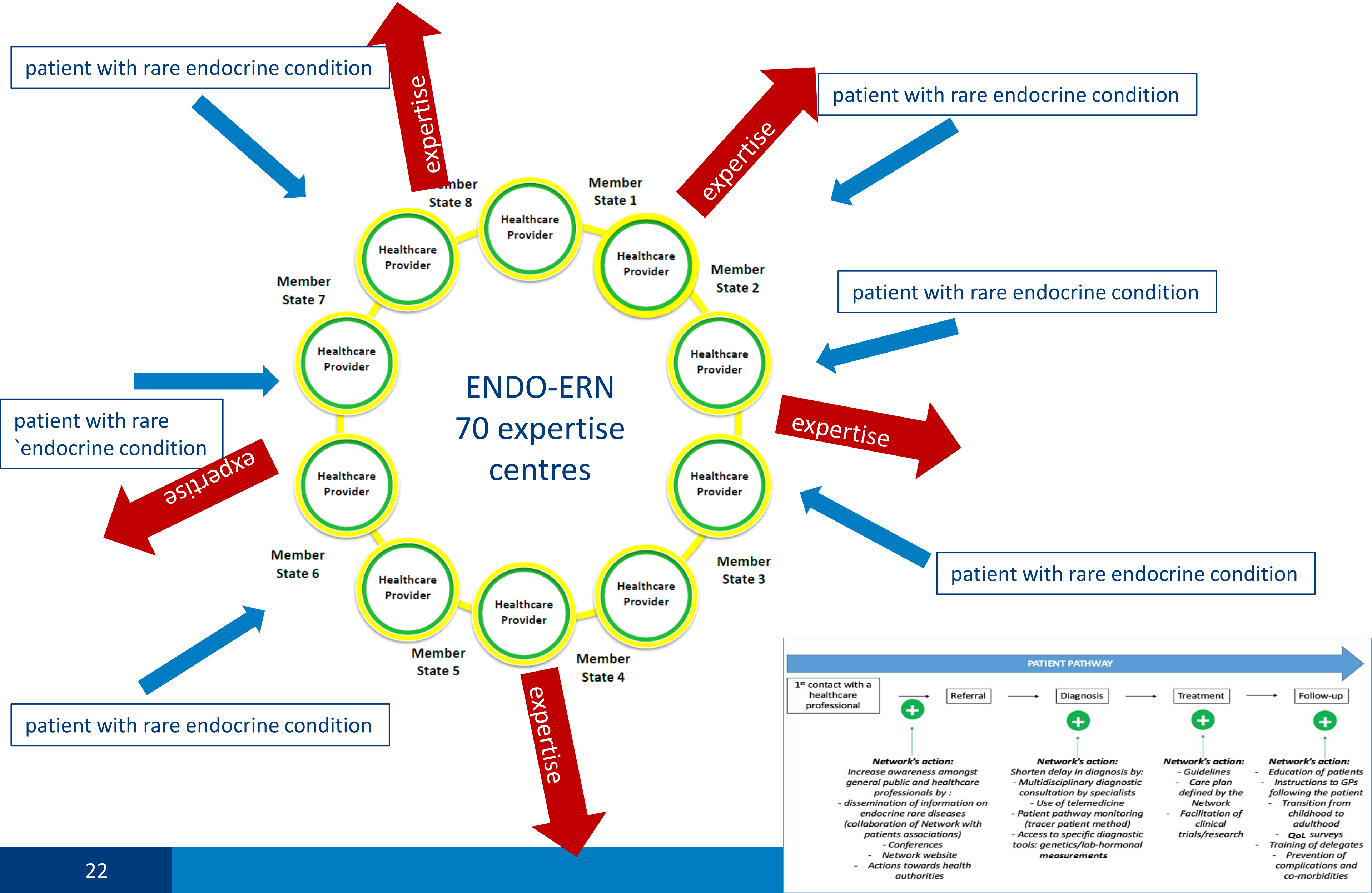
European Network of expertise centers: expertise travels to the patient!



ENDO-ERN
70 expertise centres

Healthcare Provider

Member State 1

Member State 2

Member State 3

Member State 4

Member State 5

Member State 6

Member State 7

Member State 8

patient with rare endocrine condition

patient with rare `endocrine condition

expertise

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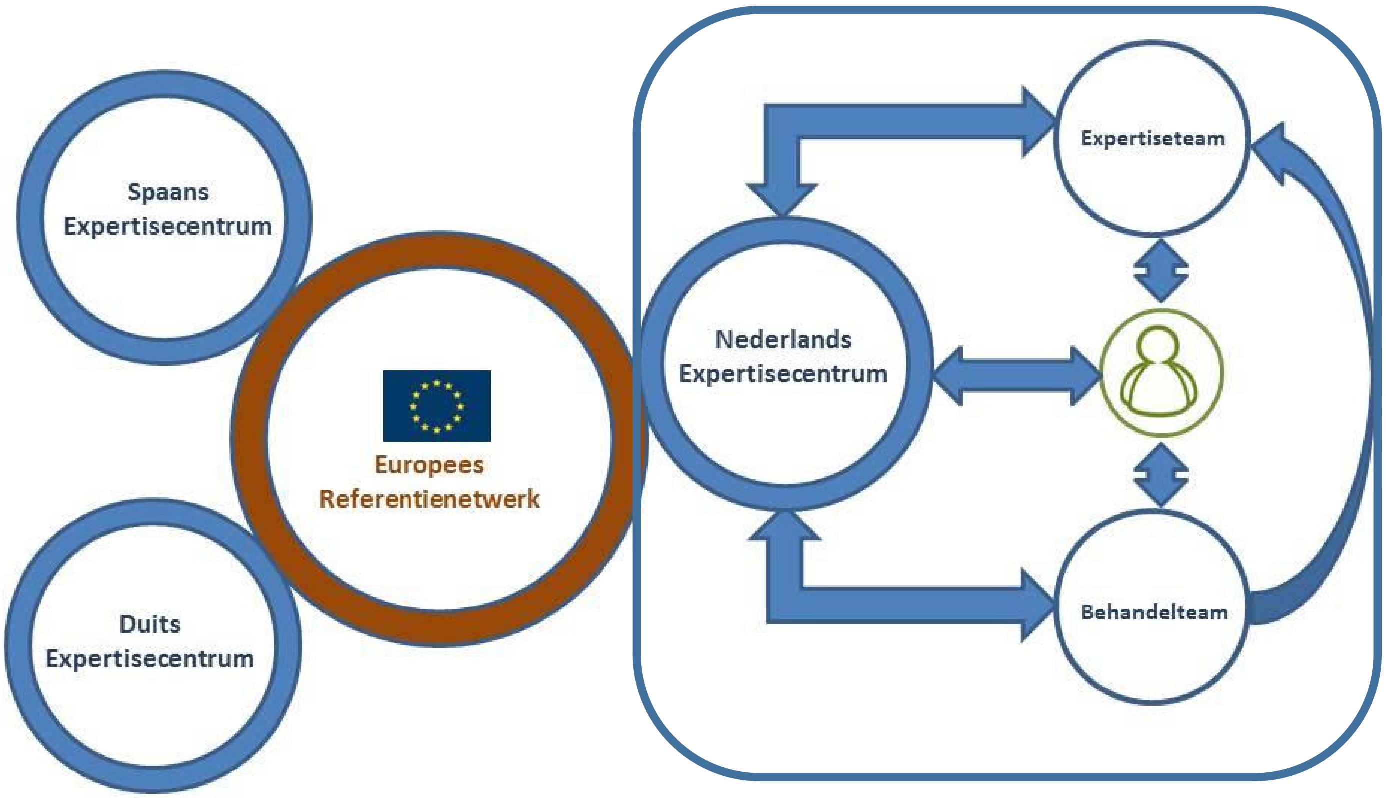
Vision document Dutch Health Ministry: integral care, centered around the patient

Multidisciplinaire organisatie voor de integrale zorg voor een (cluster van) zeldzame aandoening(en)



Visiedocument

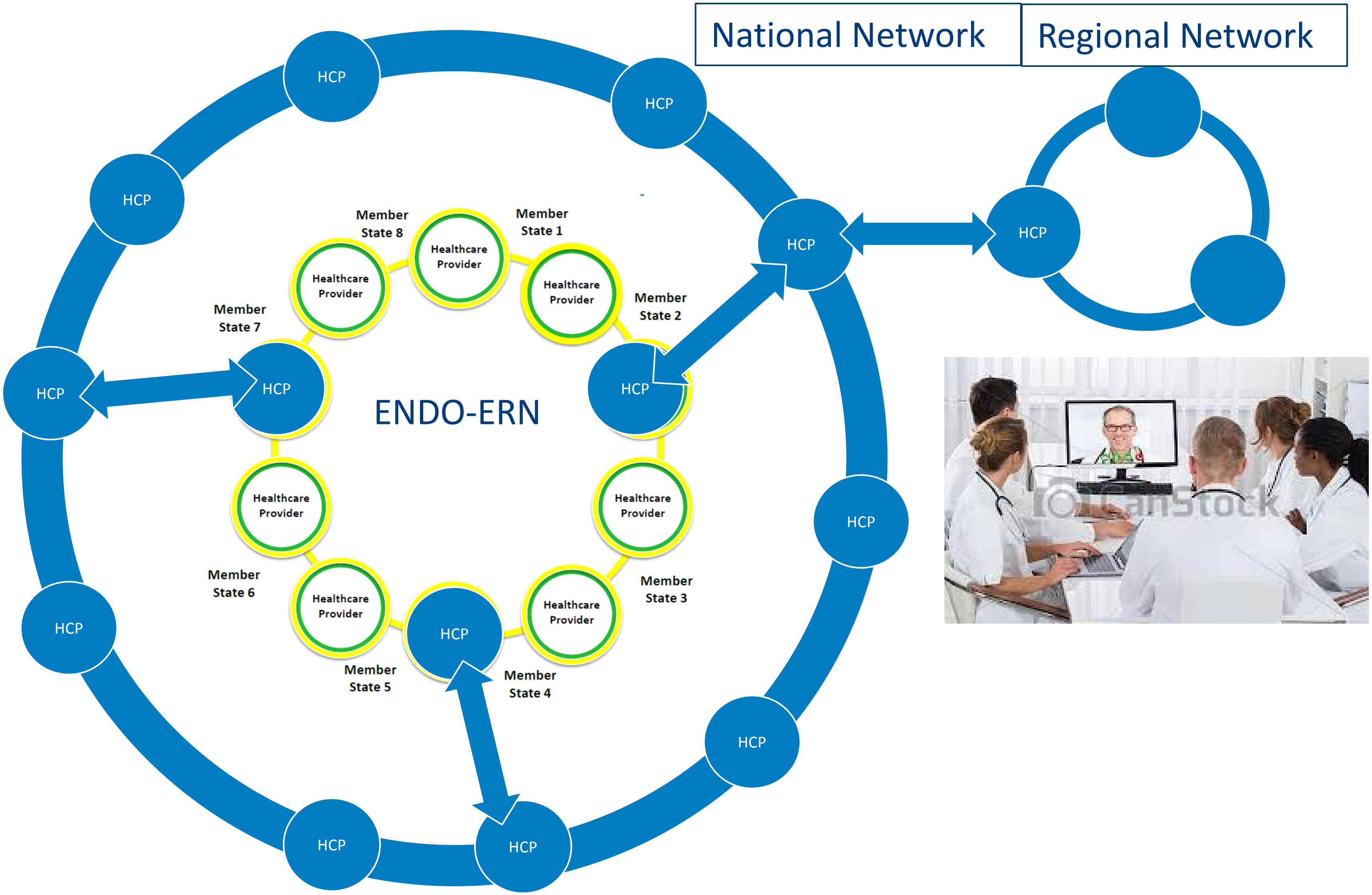
Concentratie en organisatie van zorg

bij zeldzame aandoeningen

VSOP

VOOR ZELDZAME EN GENETISCHE AANDOENINGEN

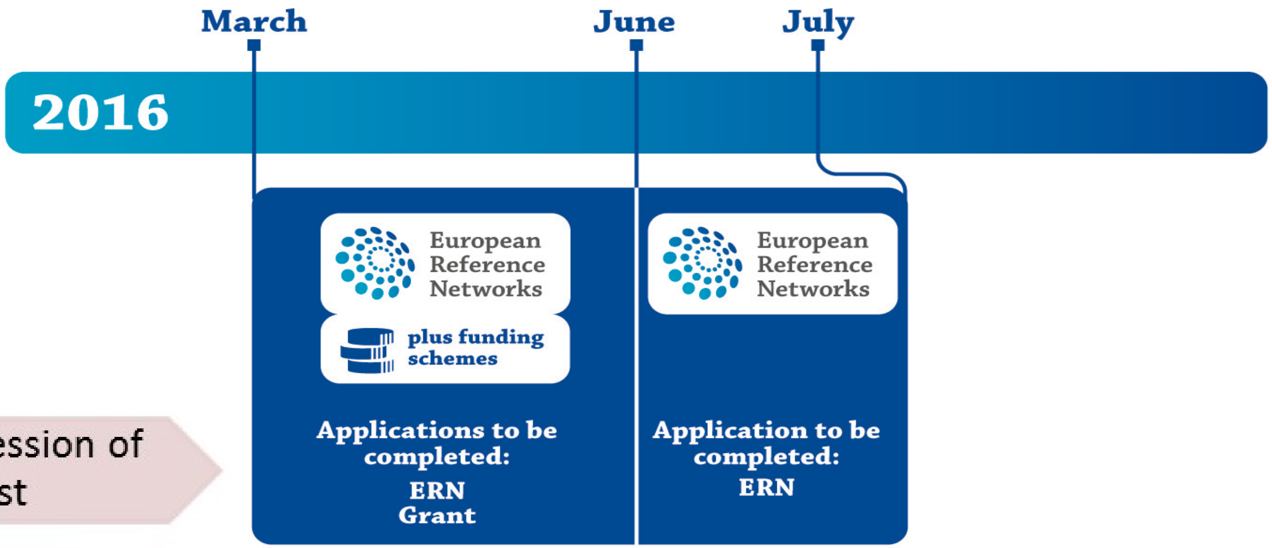
National Networks that inter-connect with regional networks and ERNs



Update on Application

European
Reference
Networks

Update on Application



European Reference Networks
plus funding schemes

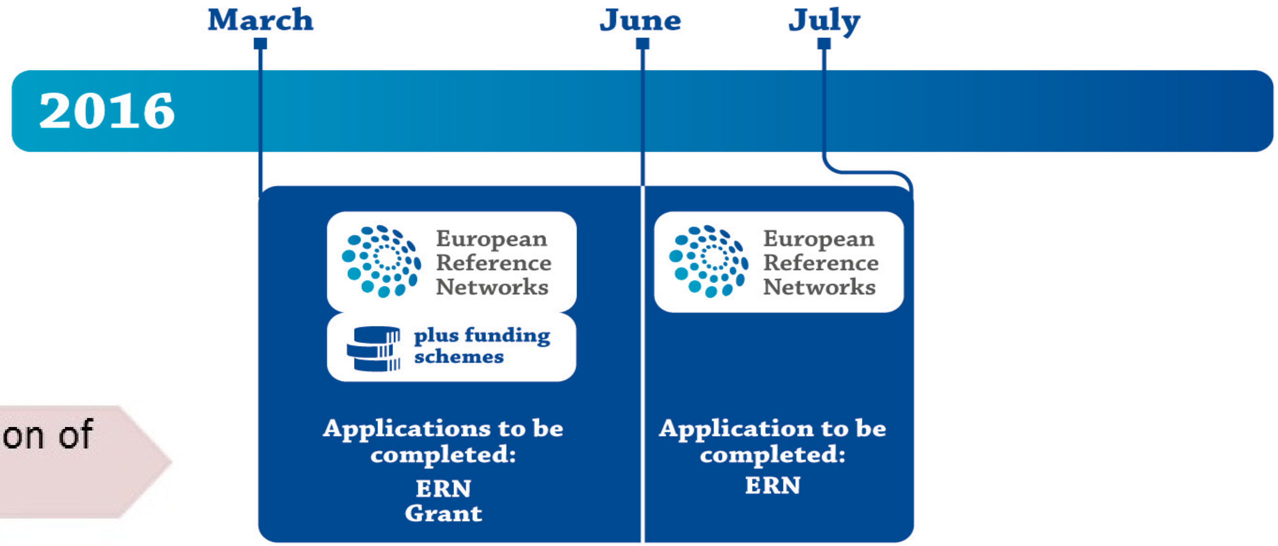
Applications to be completed:
ERN
Grant

European Reference Networks

Application to be completed:
ERN

- March - July 2016 — Call for expression of interest
- July - September 2016 — EC Eligibility check
- October - November 2016 — IAB Eligibility check & Technical assessment
- December 2016 - January 2017 — BoMS approval decision

Update on Application



March - July 2016 — Call for expression of interest

July - September 2016 — EC Eligibility check

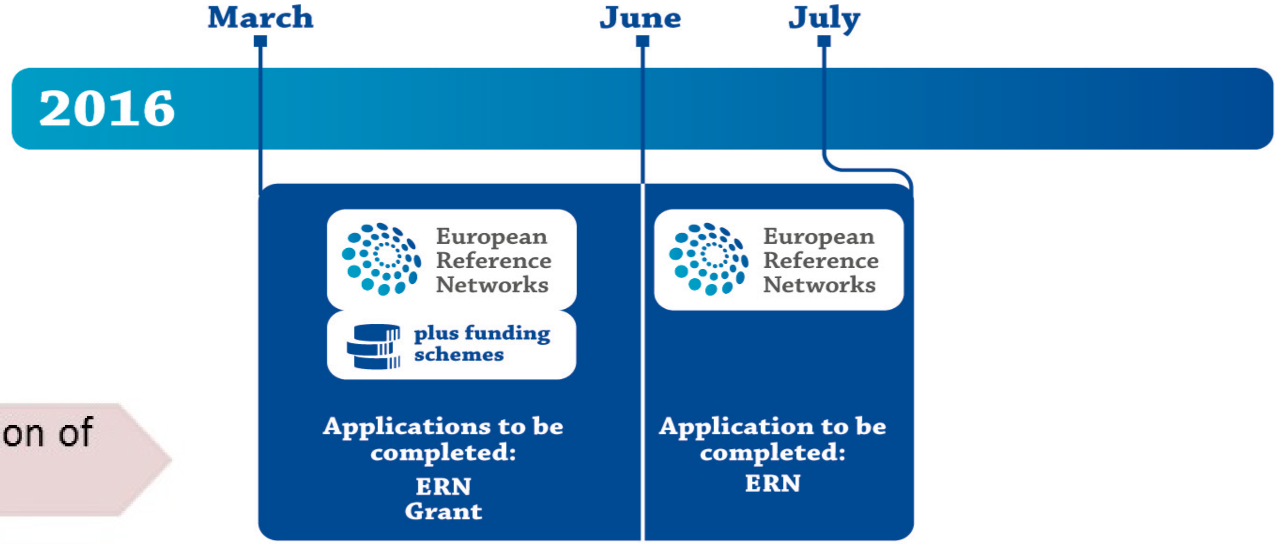
October - November 2016 — IAB Eligibility check & Technical assessment

December 2016 - January 2017 — BoMS approval decision

15/71 HCPs: full technical assessment (documentation review)
3/71 HCPs: on-site audit:
- 24 oct 2016: Tartu (Estonia) (HCP)
- 4 nov 2016: Leiden (NL) (Network + HCP)
- 7 nov 2016: Luxembourg (L) (HCP)

HCP audit:
- **patient-centered expert care (patient tracers)**
- **patient empowerment**
- **education**
- **research**

Update on Application



Applications to be completed:
ERN
Grant

Application to be completed:
ERN

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15 november 2016:
positive recommendation from IAB to the EC and BoMS for the Network and for 70/71 HCPs

Endo-ERN is ready to start!

15 dec 2016: Workshop on Registries (Glasgow, UK)

December 2016 - January 2017

BoMS approval decision

9-10 march 2017: 3rd ERN Conference: Official installation of ERNs (Vilnius, Lithuania)

27 march 2017: Endo-ERN first General Assembly (Leiden, the NL)

Initiation of operational ERN:

- update all members, advisors, national coordinators, third parties
- launch ENDO-ERN Logo & Website
- approval of Governance structure & Board Terms of Reference
- each member (HCP) signs ‘Members Role & Responsibilities’

European Reference Networks