How can ENERCA become a European Reference Network?

Joan-Lluis Vives Corrons
Lisbon, 9 October 2015
The clinical challenge

You have a Rare Anaemia

You feel ill...

Visit your doctor...

The doctor does some tests...

The doctor prescribes iron pills...

...and says: you have anaemia!

ENERCA
EUROPEAN NETWORK FOR RARE AND CONGENITAL ANAEMIAS
WHAT IS A RARE ANAEMIA?

1. KEY CLINICAL SIGN IN MORE THAN 62 RARE DISEASES
2. HEREDITARY ORIGIN IN MORE THAN 90% OF PATIENTS
3. UNKNOWN ORIGIN IN MORE THAN 10% OF PATIENTS
THE RARE ANAEMIAS IN THE CONTEXT OF RARE DISEASES

MORE THAN 500,000 CHILDREN BORN EVERY YEAR WITH A RARE ANAEMIA
THE MOST FREQUENT ARE:
1. Thalassaemia (Mediterranean Anaemia)
2. Sickle Cell Disorders (Migration Impact)

MAIN CLINICAL IMPLICATIONS

• TREATMENT (PALLIATIVE) → To reduce the severity of anaemia
  Blood Transfusions and Iron chelating

• PREVENTION → To reduce mortality and morbidity
  Newborn screening
1. ALMOST ALL HAEMATOLOGICAL DISEASES ARE RARE

2. THE TARGET ORGAN IS THE HAEMATOPOIETIC SYSTEM
   – Leukaemias, lymphomas and related neoplasms
   – Bleeding & Coagulation disorders
   – Anaemias and erythropoietic related disorders
   – White blood cell disorders leading to immune disfuncion

3. SECONDARY RARE ANAEMIAS
   Anaemias present as key clinical manifestation in non-haematological rare diseases:
   • Atypical Haemolytic Uremic syndrome (aHUS)
   • Chronic kidney anaemia (CKA)
   • Chronic liver disease (CLD)
   • Hypothiroidism
   • Inflammatory anaemia
   • Other
RARE ANAEMIAS IN THE CONTEXT OF HAEMATOLOGICAL DISEASES

THE ACTION PLAN FOR ERN

1. RARE ANAEMIAS AND RED BLOOD CELL DISORDERS
2. RARE BLEEDING & COAGULATION DISORDERS belong to EUHANET
3. RARE LEUKOCYTE DISORDERS belong to Rare Immunological and Autoinflammatory diseases
4. RARE MALIGNANT HAEMATOLOGICAL DISORDERS belong to oncological ERNs

A list of rare diseases included in the ORPHA code for Rare Haematological Diseases has been already analysed and split into different categories to be covered by the ERN. Accordingly, two different ERNs in Rare Haematological Disorders are suggested:

1. ERN in Rare Anaemias and Red Blood Cell disorders (non-oncological)
   Rare Anemias (ORPHA108997)
   Poliglobulina (ORPHA98427)

2. ERN in Rare Bleeding and Coagulation diseases (non-oncological)
   Rare coagulation disorder (ORPHA98429)
   Twin to twin transfusion syndrome (ORPHA95431)
   Congenital analbuminemia (ORPHA86816)
PATIENTS INVOLVEMENT

Patients Associations will participate through the Thalassaemia International Federation (TIF), one of the main ENERCA partners.

TIF is already part of ENERCA executive committee since 2008

Other Rare Anaemias and Related disorders patient’s Associations will join in the next future

EUROPEAN HAEMATOLOGY ASSOCIATION (EHA)

Collaboration between EHA and ENERCA for the ERN consolidación is currently analysed
11 Member States

- Belgium
- Bulgaria
- Cyprus
- France
- Germany
- Greece
- Italy
- Netherlands
- Portugal
- Spain
- UK
ENERCA progress has nourished by all the concepts and initiatives surrounding rare diseases developed in Europe along its 15 years of life.
THE FUNDAMENTAL OF ENERCA PROGRESS
(FOUR PHASES OR INDIVIDUAL PROJECTS)

Co-funded by the Health Programme of the European Union

- First network of experts
- Clear and concise information
- Protocols for diagnosis
- Congenital anaemias only

Phase II (2005 – 2008)
- Network Consolidation
- Haemoglobinopathies database
- Dissemination and awareness
- Congenital & acquired anaemias

Phase III (2009 – 2012)
- Guidelines
- Social awareness
- Patient’s empowerment
- White Book
ICT Platforms development (e-ENERCA)

Consortium & members

- e-Registry (Cyprus)
- e-Learning (France)
- Telemedicine (Belgium)

www.enerca.org

Multi channel (Computer, Smartphone, Tablet...)

Health professionals, Health authorities ... Patients
THE CHALLENGES OF ENERCA

This is not an easy job!

- **Improve** the existing **poor implementation** of comprehensive data collection for rare anaemias

  ![e-Registry]

- **Harmonise** the existing **variability** in diagnosis practices, clinical care and prevention of rare anaemias in the EU

  ![e-Learning]

- **Decrease** the existing **inequalities** for the access to high quality health care in rare anaemias between the different European Countries

  ![Telemedicine]
THE NEXT ENERCA CHALLENGE

To become an European Reference Network (ERN) on Rare Anaemias

Analyse the impact of the entry into force of the Directive 2011/24/EU on the application of patients’ rights in cross-border healthcare.
European Reference Networks (ERN): aim of Article 12: (Directive Patient's Rights to Cross border Healthcare)

Looking at best practices: MS and Centres visits

Public Consultation

Advising from:
- EUCERD
- EPAAC
- PARENT

Cross-border Healthcare Expert Group

Legal acts on ERN

Delegated Decision

- criteria and conditions for providers & Networks

Implementing Decision

- Exchange of information and expertise for ERN
- criteria for establishing and evaluating ERN

enter into force 27 May 2014

BASIC REQUIREMENTS

10 Members in at least 8 Countries of the European Union

**MEMBER**: Health care provider or CENTRE providing diagnosis, prevention and treatment

**MEMBERSHIP APPLICATION**: Individual CENTRE application
Requires a written statement from the Member State (MS) certifying that the centre accomplishes with the eligibility criteria for centres of expertise prepared by their respective National Plan or Strategy for rare diseases, as part of MS National Legislation.

**MEMBERSHIP CERTIFICATION**: This is a certificate provided by each MS for MEMBERS
If a MS cannot designate MEMBERS in its own Country, associated and/or collaborative national centres have to be designated and encourage they to cooperate with the ERN.

- Associated National Centres.
- Collaborative National Centres.
- Healthcare providers who do not fulfil the required criteria
- University and Research Centres providing knowledge and quality care tools

**In Spain**: To be part of an ERN in rare diseases, the CENTRE has to be previously certified by the National “Ministerio de Sanidad, Seguridad Social e Igualdad (MSSSI) as National Centre of Expertise called CSUR (Centres, services and Units of Reference) – On October 29, 2015, there is a meeting at the MSSSI where considerations will be established for the recognition of rare anaemias as rare haematological disorders.
BASIC REQUIREMENTS

3 Objectives (at least), out of 8 Objectives are required

1) To facilitate EUROPEAN COOPERATION on highly specialised healthcare systems

2) To contribute to the POOLING OF KNOWLEDGE

3) To facilitate CONCENTRATION OF EXPERTISE for providing high-quality healthcare

4) To maximise COST-EFFECTIVENESS by concentrating resources where appropriate

5) To reinforce RESEARCH, EPIDEMIOLOGICAL SURVEILLANCE AND TRAINING

6) To facilitate MOBILITY OF EXPERTISE to develop and share knowledge and best practices

7) To encourage the DEVELOPMENT OF QUALITY AND SAFETY BENCHMARKS

8) To provide ACCESS TO HEALTH SERVICES for all patients with RD wherever they live
Objective 4: MAXIMISE THE COST-EFFECTIVE USE OF RESOURCES

a) e-ENERCA WP4
   e-registry platform of patients for shaping policies addressing specific needs to concentrate resources

b) ENERCA WITE BOOK
   Centers of Expertise (CoE) recommendations on technical, ethical and patient’s expectations

Objective 6: FACILITATE MOBILITY OF EXPERTISE

a) ENERCA 3:
   Good Practices (GP) recommendations on diagnosis, prevention and clinical management

b) e-ENERCA WP5:
   e-learning platform for continuous medical education – Courses and Symposia

c) e-ENERCA WP6:
   Telemedicine platform for sharing knowledge, expertise, and clinical management
A position paper and the most important ENERCA outcome after 10 years of experience for the creation of a European Reference Network (ERN) in rare anaemias.

It contains the essential specific criteria to be considered in the process of identification and recognition of Centres of Expertise as healthcare providers.

Centres of Expertise will be the nodes of the future ERN, that will act as a focal point for information, medical training, research, and dissemination of rare anaemias.
Research and Innovation
Promote collaborative projects in both, preclinical and clinical fields
Objectives 1 and 5

Telemedicine
Provide an e-Health tool to facilitate remote consultations on diagnosis and clinical management
Objectives 6 and 8

Dissemination of knowledge and expertise
Improve training and disseminate knowledge and expertise
Objectives 5 and 6

Guidelines and harmonization of procedures
Promote the harmonization of procedures and standards for clinical management and diagnosis
Objective 7

Evaluation
Evaluate the functioning of the net and identify needs through a set of indicators: Objectives, process, results and outcomes

Diagnosis and prevention
Promote primary, secondary and tertiary prevention, improve diagnosis and its availability across Europe
Objectives 2, 3, 4 and 8

Epidemiological surveillance and allocation of resources
Mapping patients and facilities to identify needs where appropriate
Objectives 4, 5 and 6

Coordination
Governance and coordination of the activities of the net, the communication in/out the net, contact to EC, MS and third parties

Work Teams

Organization Chart

ERN FOR RARE ANAEMIAS - ORGANISATION CHART
ENERCA ERN Governance

ENERCA Advisory Board

ENERCA Executive Board

ENECA Coordinator

Head of Node

WT 1 Diagnosis and prevention

WT 2 Epidemiological surveillance and allocation of resources

WT 3 Guidelines and harmonization of procedures

WT 4 Dissemination of knowledge and expertise

WT 5 Telemedicine

WT 6 Research and Innovation

WT 7 Coordination

WT 8 Evaluation

Advise
ENERCA  Coordination Team

Head of Project – Joan-Lluis Vives Corrons (jl.vives@clinic.ub.es.org)
Project Manager - María del Mar Mañú Pereira (maria.manu@enerca.org)
Project Web manager- Victoria Gutiérrez (victoria.gutierrez@enerca.org)
Project Assistant – Patricia Carvajal (pkcarvajal@clinic.ub.es)

Red Cell Pathology Unit
Hospital Clinic i Provincial University of Barcelona
c/ Villarroel 170
080036-Barcelona
Phone: +34 934515950
enerca@enerca.org

www.enerca.org