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# ENERCA 3

European Reference  
Network of Expert Centers  
in Rare Anaemias

ENERCA is a project in the Health Programme 2008  
of the European Commission



## Project background

The **ENERCA Project** (European Network for Rare and Congenital Anaemias) started in October 2002 and ended in April 2004 (ENERCA 1). It allowed the set up of an officially endorsed website ([www.enerca.org](http://www.enerca.org)) and restricted access areas to professionals. This tool was designed to:

- Provide patients, their relatives, and care providers with **clear and concise information**, in their own language, about the different rare congenital anaemias known so far.
- Provide physicians with a **rapid protocol** for the identification and diagnosis of rare congenital anaemias.

The **second ENERCA Project** (ENERCA 2) (September 2005-August 2008), in addition to congenital anaemias also covered all rare causes of anaemia, whether hereditary or acquired. The main results of this second phase were:

- The development of a pilot study to initiate the **mapping of existing expert centres** for rare anaemias in Europe.
- The creation of a personal **medical alert card** as an aid for a prompt response in case of emergency.
- The creation of a **database collection of haemoglobinopathies** as the basis for the future development of the European registry of rare anaemias.
- The **promotion of the neonatal screening of haemoglobinopathies** in those European countries without existing epidemiological information. This has led to the knowledge of a prevalence of haemoglobinopathies in Catalonia and Latvia.
- The **dissemination of ENERCA knowledge** in Europe by providing an online forum and the organisation of an annual European symposium on rare anaemias.
- The establishment of a quality assessment pilot study for **red blood cell morphology and quantification** of haemoglobins A2 and F.
- The publication of ENERCA newsletters on relevant aspects of the management of the rare anaemias.
- **Consolidation of the website:** [www.enerca.org](http://www.enerca.org)

## ENERCA 3: Objectives and outcomes

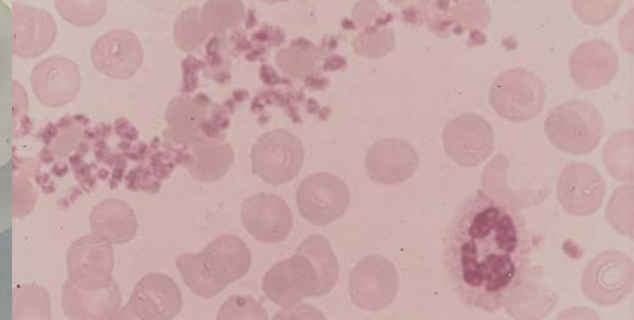
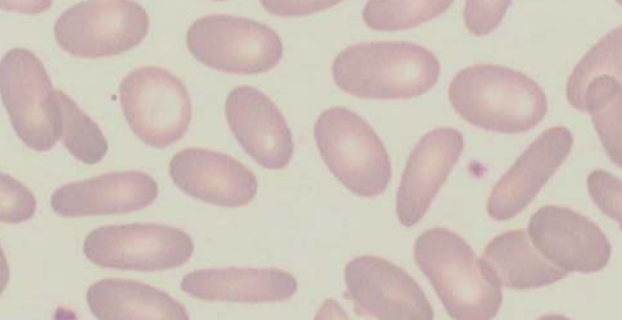
The main objective of the third ENERCA project is the establishment of a European Reference Network of Expert Centres in Rare Anaemias. The Network will link the existing European centres, and will become a platform for providing information and services to health professionals, patients, citizens, stakeholders interested in rare diseases, authorities and pharmaceutical industry managers. It will also help to promote the harmonisation of diagnostic procedures, the provision of a tool for epidemiological surveillance of rare anaemias in Europe and cooperation between experts. Its final objective is to offer health professionals and patients easy access to high-quality information on rare anaemias, useful for specific healthcare services in all Member States.

ENERCA 3 outcomes will include:

- The establishment of a **European Reference Network** in rare anaemias.
- The preparation of a comprehensive **catalogue for external quality assessment of procedures** used in the diagnosis of rare anaemias.
- A better knowledge of the status of rare anaemias in the Member States through comparable epidemiological European data.
- The publication of guidelines for the harmonisation of diagnostic and/or therapeutic procedures in rare anaemias.
- An increase in health professionals' knowledge of rare anaemias (training courses and symposiums).
- Increased social awareness of rare anaemias by means of educational material in the relevant languages.



» *The achievement of the general objectives and outcomes will contribute to the improvement of health and quality of life of patients with rare anaemias by increasing the efficacy of patient diagnosis, treatment and follow-up.*



## ENERCA, a tool for improving the current situation

The aims of ENERCA are closely linked to the strategic objectives of the EU Health Programme 2008-2013. It is particularly focused on reducing health inequalities across the European Union, and on the Community added value to national efforts at tackling rare diseases through a Europe-wide network for the exchange of knowledge and expertise.

The project also relates to one of the main objectives of the Commission's Work Plan for 2008, "putting citizens first". It includes improving patient safety and the quality of health services. National and local patients' organisations have a key role to play in disseminating information and promoting patient empowerment. The ENERCA web and the training initiatives will provide a meeting place for them.

## European added value

Preventing disease and promoting policies that lead to a healthier way of life is important for the well-being of European Union citizens. Accordingly, common principles in all European Union health systems must ensure clarity and confidence with regard to authorities setting and monitoring healthcare standards. For these two pivotal aspects, a specific framework for cross-border healthcare and European cooperation on health services should be developed. This cooperation has two main objectives: helping health professionals and improving patient care.

## Strategic impact

The main benefits of a European Reference Network of expert centres in rare anaemias will be:

- Easy access to expert centres throughout the European Member States.
- Comparable data on patients suffering from rare anaemias, and epidemiological surveillance throughout Europe.
- Establishment of common treatment protocols and dissemination of information about rare anaemias to improve the awareness of these rare diseases among health professionals and the general public.

All these benefits, together with the promotion of scientific activities (symposiums, workshops and others) and close cooperation with other associations and stakeholders (public institutions, public health authorities pharmaceutical companies and others) guarantee ENERCA's sustainability as a recognised health services provider after 2012.



» The development of ENERCA 3 will benefit patients with rare anaemias, but will also be relevant in maintaining and increasing the reputation of European expertise in the diagnosis and management of these disorders.

» As with other rare diseases, because of the reduced number of patients with rare anaemias and the need to mobilise resources, their study can only be efficient if done in a coordinated, European way.

» According to the European Commission, a disease is rare when its population frequency is less than 5 cases in 10,000 individuals. To date, some 90 different kinds of rare anaemias have been identified and listed by ENERCA.

# Work packages

## WP1

### NETWORKING OF EXPERT CENTRES

#### OBJECTIVES

- To analyse the ethical and legal issues in each European country for the management of patients and samples for diagnostic purposes.
- To define the criteria that an expert centre has to fulfill in order to be included in the European Reference Network of Expert Centres in Rare Anaemias.
- To create a European Reference Network of Expert Centres in Rare Anaemias

#### DELIVERABLES

- Legal and ethical items: questionnaire to be sent to centres, and scheme for national reports.
- Pan-European comparative analysis of ethical and legal framework for referral of patients and samples.
- Legal and ethical report.
- White Book for the creation of the European Reference Network of Expert Centres in rare anaemias.

## WP2

### QUALITY OF PATIENT CARE

#### OBJECTIVES

- To improve the quality of laboratory data on rare anaemias.
- To facilitate the participation of expert centres in external quality assurance.
- To provide educational external quality assurance schemes exercises.
- To prioritise the preparation of guidelines for the laboratory diagnosis of rare anaemias.
- To develop a European registry of rare anaemias.

#### DELIVERABLES

- Questionnaire to survey the available accredited external quality assurance schemes relevant to the rare anaemias.
- Comprehensive catalogue of available external quality assessment.
- Guidelines for the laboratory diagnosis of rare anaemias.
- Protocol for the development of a European Registry of rare anaemias.
- Reports on epidemiological data and educational external quality assurance schemes results.

## WP3

### EDUCATION AND TRAINING

#### OBJECTIVES

- To disseminate up-to-date knowledge on rare anaemias to European health professionals, practitioners and patients by establishing collaboration with recognised European organisations, professional education and training activities.
- Health education actions aimed at medical professionals, primary care personnel, patients and their families. These will be led by: the ENERCA website ([www.enerca.org](http://www.enerca.org)), the European School of Haematology (ESH), the European Haematology Association (EHA) and the International Society of Haematology (ISH), and the World Health Organization (WHO). For patients and their families this will be done in close collaboration with the Thalassaemia International Federation (TIF).

#### DELIVERABLES

- Report on the training courses:
  - Thalassaemia and other haemoglobin disorders.
  - Very rare red blood cell disorders: diagnosis of membrane defects, enzymopathies, congenital dyserythropoietic anaemias (CDA) and other rare red blood cell diseases.
  - Constitutional iron disorders leading to chronic anaemia.
- Conferences on haemoglobinopathies, RBC membrane and enzyme pathologies for health professionals and patients. The sessions will be recorded and distributed in video format.

## Project management

## WP7 EVALUATION OF THE PROJECT

- Monitoring the general management of the Project and evaluating its progress; that is to say, the achievement of objectives, the outcomes and the impact of the Project's results on the improvement in the health service of patients with rare anaemias, and on the health services providers, authorities, industry and any other stakeholders.
- Evaluation programme for detecting partial progress failures or weak points in order to implement corrective measures.

## WP8 DISSEMINATION OF THE RESULTS, AND FUTURE SUSTAINABILITY

- To get the critical mass of interest necessary for the project's success.
- To organise two European Symposia on Rare Anaemias.
- To consolidate the ENERCA website and its services.
- To assure Project sustainability after 2012.

## WP4

### PUBLIC HEALTH ISSUES AND MANAGEMENT OF PATIENTS WITH SICKLE CELL DISORDERS

#### OBJECTIVES

- To define the technical criteria necessary to become a sickle cell disease expert centre and to identify the European expert centres.
- To promote collaboration in the epidemiological registry of patients with sickle cell disease throughout Europe; to set up a programme for epidemiological surveillance.
- To prepare guidelines and recommendations on prevention, diagnosis and treatment, if feasible.
- To improve the knowledge of sickle cell disease among professionals, patients and patients associations.

#### DELIVERABLES

- Standards for the clinical care of children and adults with sickle cell disease; definition of facilities needed to assume that clinical care.
- Picture of the Sickle Cell Disease expert centres existing in Europe and the facilities available at those centres.
- Guidelines on: neonatal screening, prenatal screening and antenatal diagnosis; follow-up of children and adult patients with sickle cell disease; management of transfusions; acute events; and pregnancy.
- Interactive CD/e-learning system on sickle cell disease for professionals, and on prevention for the public.

## WP5

### PUBLIC HEALTH ISSUES AND MANAGEMENT OF PATIENTS WITH THALASSAEMIA

#### OBJECTIVES

- To define the technical criteria necessary to become a thalassaemia expert centre and to identify the European expert centres.
- To define the indicators and to collect data for establishing epidemiological mapping and registry of patients affected by thalassaemia throughout Europe.
- Guidelines and recommendations on prevention, diagnosis and treatment of thalassaemia.
- To improve the knowledge of thalassaemia among health professionals through educational courses and e-learning resources in collaboration with WP3, and among patients and society in general, through educational material and by promoting relations with patients associations.

#### DELIVERABLES

- Enquiry based on the consensus criteria included in the White Book: technical, legal and ethical criteria necessary to become a thalassaemia expert centre. Picture of the thalassaemia expert centres that exist in Europe and their facilities.
- Guidelines on prenatal screening, antenatal diagnosis, genetic counselling and clinical management of thalassaemia.
- Educational material for patients with thalassaemia.
- Report on twinning and collaborative research initiatives.

## WP6

### PUBLIC HEALTH ISSUES AND MANAGEMENT OF PATIENTS WITH VERY RARE ANAEMIAS

#### OBJECTIVES

- To define the technical criteria necessary to become a Very Rare Anaemia expert centre and to identify the European expert centres.
- To promote collaboration in the epidemiological registry of patients with rare anaemias.
- To enable clinicians to rapidly identify the defects of red cells and their precursor cells underlying the Very Rare Anaemias, and promote the appropriate treatment.
- To support access of all citizens of the European Union Member States to timely diagnosis and treatment, and the exchange of best practice for patients groups.

#### DELIVERABLES

- Enquiry based on the consensus criteria included in the White Book: technical, legal and ethical criteria necessary to become a Very Rare Anaemias expert centre.
- Picture of the European Very Rare Anaemias expert centres and their facilities.
- Guideline on the strategies to recognise and confirm the exact diagnosis of Very Rare Anaemias and recommendations for management.
- Educational material for patients.
- Reports on the harmonisation of procedures for diagnosis, treatment and follow-up of patients with Very Rare Anaemias and the results of research cooperation.

## VIABILITY OF THE PROJECT

## WP9 COORDINATION OF THE PROJECT

- Project management, quality assurance and assessment of progress and results.
- To guarantee compliance with the work plan and to achieve real coordination.

# The consortium

The project will be carried out by 48 partners, 24 of whom are associated partners and the other 24 collaborating partners, covering the vast majority of Member States. Most of the partners have been working together since 2002 and all of them are well known and recognised experts in their respective fields. A solid and professional management structure has been created to allow the smooth running of the project, with each WP leader coordinating his/her activities. All partners have expressed their full commitment to the project and will be contributing the necessary resources.



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For further information:  
[www.enerca.org](http://www.enerca.org)

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