The 6th European Symposium on Rare Anaemias is an activity of the ENERCA project which aims to disseminate up-to-date knowledge and increase the public awareness about congenital and rare anaemias. As in other rare diseases, the recognition of expert centres, promotion of best practices and education and training of multidisciplinary health professionals teams are basic in order to serve patient needs, both in diagnosis and case management. Patients are always at the heart of ENERCA policies, like those of the Multi-ethnic organisation for patients with Sickle Cell and Thalassemia (OSCAR), Belgic Association of Thalassaemia (ASBL), Aplastic Anaemia and Paroxysmal Nocturnal Hemoglobinuria (PNH) - rare blood disease community (HematosLife), Pyruvate Kinase Deficiency (PKD) Support Group, and Thalassaemia International Federation (TIF).

In this respect, and specifically integrated in the framework of the 6th European Symposium on Rare Anaemias, the three organizers, ENERCA, UMCU and ERASME, with the support of OSCAR, ASBL, HematosLife, PKD Support Group and TIF have collaborated in setting up the 1st Dutch-Belgian meeting for patients and health professionals in Amsterdam.

This year, transversal topics centered on common medical problems of patients with sickle cell, thalassaemia and other forms of haemolytic anaemia will be one of the key points of the symposium. These plenary sessions will deal with multiple converging lectures as well as with the state of the art therapy treatments and innovations in the field.

The second undeniable cornerstone for this symposium will be the special focus on the very rare anaemias, dealing intensively with PKD and PNH prevention, diagnosis, treatment and management. Furthermore, interactive sessions between patients and worldwide experts will develop a perfect atmosphere for brainstorming and sharing doubts among the attendants.

**6th European Symposium on Rare Anaemias**

1st Dutch-Belgian meeting for patients and health professionals

**ENERCA**

The European Network for Rare and Congenital Anaemias (ENERCA), a project co-funded by the European Commission was set up in 2002 to help medical professionals and patients with rare anaemias by improving updated information and public health services through its website. The main goal is assuring the same level of access for both health professionals and patients independently of their country of practice or origin and to decrease health inequalities in the field of rare anaemias. The new project e- ENERCA (2013-2016) will promote the recognition of Centres of Expertise at national level, a cornerstone as the nodes of the future European Reference Network (ERN) in Rare Anaemias to be implemented by the EU in the next future. Moreover, the new e-Health tool will include three main platforms a) electronic health records (EHR), for epidemiological surveillance, b) e-learning for on-line education and training and c) telemedicine for sharing experiences, contribute to an early diagnosis and facilitate chronic anaemia patient’s care across Europe.

www.enerca.org
SATURDAY 21\textsuperscript{th} November 2015

09:00-09:30 REGISTRATION

09:30-11:00 WELCOME SESSION
Chairperson: Richard van Wijk
European Network for Rare and Congenital Anaemias – Telemedicine
Joan Lluis Vives Corrons
Dutch National Platform for Rare Diseases
Elfriede Swinnen
European Commission – Rare Diseases
Enrique Terol

09:30-11:00 WELCOME SESSION
Chairperson: Richard van Wijk
European Network for Rare and Congenital Anaemias – Telemedicine
Joan Lluis Vives Corrons
Dutch National Platform for Rare Diseases
Elfriede Swinnen
European Commission – Rare Diseases
Enrique Terol

11:00-11:30 COFFEE BREAK

11:30-13:00 PLENARY SESSION
Common medical problems of patients with sickle cell, thalassaemia and other forms of haemolytic anaemia - I
Chairperson: Eduard van Beers
Domenica Cappellini
Vascular effects of hemolysis
Gregory Kato
Will your child be sick as well? Testing before pregnancy
Béatrice Gulbis

13:00-14:30 LUNCH

14:30-15:30 PLENARY SESSION
Pyruvate kinase deficiency (PKD)
Chairperson: Richard van Wijk
Introduction to PKD
Richard van Wijk
Activator treatment for PKD – Results from Phase 1 and overview of the Phase 2 trial
Sam Agresta
PKD National History Study
Rachel Grace

15:30-16:30 BREAK

16:30-17:00 PLENARY SESSION
Common medical problems of patients with sickle cell, thalassaemia and other forms of haemolytic anaemia - II
Chairperson: Bart Biemond
Hormone related problems (Endocrinopathies and osteoporosis)
Vincenzo de Sanctis
Dyspnea
Eduard van Beers
Kidney dysfunction (Renal failure)
Swee Lay Thein

17:30-18:30 POSTER WALK

19:00-22:00 GALA DINNER
(Not included in the registration fee)
SUNDAY 22<sup>nd</sup> November 2015

09:00-10:30  **PLENARY SESSION**
Common medical problems of patients with sickle cell, thalassaemia and other forms of haemolytic anaemia - III
Chairperson: Béatrice Gulbis
Transition from paediatrics to adulthood: Marjon Cnossen
Gene therapies in rare anaemias: Jose Carlos Segovia
New genetic tests, helpful or a waste of money?: Patricia Aguilar-Martinez
EU Registry: Michael Angastiniotis

10:30-11:00  **COFFEE BREAK**

11:00-12:30  **PLENARY SESSION**
Paroxysmal Nocturnal Hemoglobinuria (PNH)
Chairperson: Lucio Luzzatto
Introduction to PNH: Sacha Zeerleder
PNH and complement activation: Marije Bartels
PNH in childhood: Marije Bartels
TPH/HUS: To be confirmed

12:30-14:00  **LUNCH**

14:00-14:30  **HERMANN HEIMPEL**
HONORARY LECTURE
Achille Iolascon

14:30-16:00  **PLENARY SESSION**
Comprehensive care for patients with sickle cell, thalassaemia and other forms of haemolytic anaemia – IV
Chairperson: Patricia Aguilar-Martinez
Psychosocial effects of rare anaemias: Patricia Aguilar-Martinez
Patient perspective: Michael Angastiniotis
Role of nurses in treating patients with rare anaemias: Nurse contact group
Telemedicine and tele-expertise for rare anaemias: Béatrice Gulbis
Research and clinical trials, what are the rights of patients?: Pilar Nicolas

16:00-16:15  **CLOSURE OF THE SYMPOSIUM**
Joan LLuis Vives Corrons

**Poster Abstract Submission Deadline:** September 30th, 2015
Conference venue
The symposium will be held in:
PLANETARIUM AMSTERDAM
Kromwijkdreef 11
1108 JA Amsterdam
020 651 8585

Meeting registration
For health professionals:
Early registration (before 15th October 2015): 150€
Late registration (after 15th October 2015): 200€
For medical students registration fee: 100€
For patients and relatives the registration is free

OFFICIAL LANGUAGES
Dutch & English / Simultaneous interpretation will be available for the whole event

Poster submission
If you are willing to participate in the poster session please, send your ABSTRACT Communication up to 250 words no later than 30th September 2015 to the Technical Secretary (vgutierrez@clinic.ub.es)
Organizing committee

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Michael Angastiniotis - Thalassaemia International Federation
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