6th EUROPEAN SYMPOSIUM ON RARE ANAEMIAS

1st Dutch-Belgian meeting for patients and health professionals

21th -22th November 2015
Amsterdam • The Netherlands

*EHA-CME accreditation requested
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 www.enerca.org. 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.
# SATURDAY 21<sup>th</sup> November 2015

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
</tr>
</thead>
<tbody>
<tr>
<td>09:00-09:30</td>
<td><strong>REGISTRATION</strong></td>
</tr>
<tr>
<td>09:30-11:00</td>
<td><strong>WELCOME SESSION</strong></td>
</tr>
<tr>
<td></td>
<td>Chairperson: María del Mar Mañú</td>
</tr>
<tr>
<td></td>
<td>European Network for Rare and Congenital Anaemias – Telemedicine</td>
</tr>
<tr>
<td></td>
<td>Joan Lluis Vives Corrons</td>
</tr>
<tr>
<td></td>
<td>Dutch National Platform for Rare Diseases</td>
</tr>
<tr>
<td></td>
<td>Paul Boom</td>
</tr>
<tr>
<td></td>
<td>Belgian Platform for Rare Diseases (Belgian Authority for rare disease plan)</td>
</tr>
<tr>
<td></td>
<td>Elfriede Swinnen</td>
</tr>
<tr>
<td></td>
<td>European Commission – Rare Diseases</td>
</tr>
<tr>
<td></td>
<td>Jaroslaw Waligora</td>
</tr>
<tr>
<td>11:00-11:30</td>
<td><strong>COFFEE BREAK</strong></td>
</tr>
<tr>
<td>11:30-13:00</td>
<td><strong>PLENARY SESSION</strong></td>
</tr>
<tr>
<td></td>
<td><strong>Common medical problems of patients with sickle cell, thalassaemia and other forms of haemolytic anaemia- I</strong></td>
</tr>
<tr>
<td></td>
<td>Chairperson: Eduard van Beers</td>
</tr>
<tr>
<td></td>
<td>Iron overload and chronic blood transfusion</td>
</tr>
<tr>
<td></td>
<td>Samantha Benghiat</td>
</tr>
<tr>
<td></td>
<td>Vascular effects of hemolysis</td>
</tr>
<tr>
<td></td>
<td>Gregory Kato</td>
</tr>
<tr>
<td></td>
<td>Will your child be sick as well? Testing before pregnancy</td>
</tr>
<tr>
<td></td>
<td>Béatrice Gulbis</td>
</tr>
<tr>
<td>13:00-14:30</td>
<td><strong>LUNCH</strong></td>
</tr>
<tr>
<td>14:30-16:00</td>
<td><strong>PLENARY SESSION</strong></td>
</tr>
<tr>
<td></td>
<td><strong>Pyruvate kinase deficiency (PKD)</strong></td>
</tr>
<tr>
<td></td>
<td>Chairperson: Richard van Wijk</td>
</tr>
<tr>
<td></td>
<td>Introduction to PKD</td>
</tr>
<tr>
<td></td>
<td>Richard van Wijk</td>
</tr>
<tr>
<td></td>
<td>Activator treatment for PKD – Results from Phase 1 and overview of the Phase 2 trial</td>
</tr>
<tr>
<td></td>
<td>Ann Barbier</td>
</tr>
<tr>
<td></td>
<td>PKD National History Study</td>
</tr>
<tr>
<td></td>
<td>Rachael Grace</td>
</tr>
<tr>
<td>16:00-16:30</td>
<td><strong>BREAK</strong></td>
</tr>
<tr>
<td>16:30-18:00</td>
<td><strong>PLENARY SESSION</strong></td>
</tr>
<tr>
<td></td>
<td><strong>Common medical problems of patients with sickle cell, thalassaemia and other forms of haemolytic anaemia-II</strong></td>
</tr>
<tr>
<td></td>
<td>Chairperson: Bart Biemond</td>
</tr>
<tr>
<td></td>
<td>Hormone related problems</td>
</tr>
<tr>
<td></td>
<td>(Endocrinopathies and osteoporosis)</td>
</tr>
<tr>
<td></td>
<td>Vincenzo de Sanctis</td>
</tr>
<tr>
<td></td>
<td>Dyspnea</td>
</tr>
<tr>
<td></td>
<td>Eduard van Beers</td>
</tr>
<tr>
<td></td>
<td>Kidney dysfunction (Renal failure)</td>
</tr>
<tr>
<td></td>
<td>Swee Lay Thein</td>
</tr>
<tr>
<td>18:00-18:45</td>
<td><strong>POSTER WALK</strong></td>
</tr>
<tr>
<td>19:00-22:00</td>
<td><strong>BUFFET GALA DINNER</strong></td>
</tr>
<tr>
<td></td>
<td>(Not included in the registration fee)</td>
</tr>
</tbody>
</table>
SUNDAY 22\textsuperscript{th} 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 anemias
Jose Carlos Segovia
New genetic tests, helpful or a waste of money?
Patricia Aguilar-Martinez
EU Registry
Michael Angastiniotis

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
Patient perspective
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

10:30-11:00 COFFEE BREAK

11:00-12:30 PLENARY SESSION
Paroxysmal Nocturnal Hemoglobinuria (PNH)
Chairperson: Rosario Notaro
Introduction to PNH
Rosario Notaro
PNH and complement activation
Sacha Zeerleder
PNH in childhood
Marije Bartels
TTP/HUS
Nicole van de Kar

12:30-14:00 LUNCH

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

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

Poster Abstract Submission Deadline: November 6\textsuperscript{th}, 2015
Conference venue
The symposium will be held in:

**PLANETARIUM AMSTERDAM**
Kromwijkdreef 11
1108 JA Amsterdam
020 651 8585

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

**Meeting registration**
For health professionals:
- Early registration (before 15th October 2015): 150€
- Late registration (after 15th October 2015): 200€

For physicians in training registration fee: 100€

For patients and relatives (up to two family members) the registration is free

**Buffet Gala dinner** is not included in the registration fee: 50€

**Hotel reservation**
Reservations have to be self-made by attendants. There are three wonderful hotel options available. Choose the one you prefer, complete the Hotel Registration Form (available in [http://www.enerca.org/symposium2015](http://www.enerca.org/symposium2015)), and send it by mail or email to Planetarium Amsterdam (Subject ENERCA): e-mail: info@planetariumamsterdam.nl Phone +31-20-651 8585

**REGISTRATION**
Go to [http://www.enerca.org/symposium2015](http://www.enerca.org/symposium2015) and complete the online registration form.

**POSTER SUBMISSION**
If you are willing to participate in the poster session please, send your ABSTRACT Communication up to 250 words no later than 6th November 2015 to the Technical Secretary (v.gutierrez@clinic.ub.es)
Organizing committee

Joan Lluis Vives (ENERCA)
Mar Mañú (ENERCA)
Victoria Gutiérrez (ENERCA)
Richard van Wijk (UMCU)
Eduard van Beers (UMCU)
Béatrice Gulbis (ERASME)

Technical secretary

Victoria Gutiérrez
vgutierrez@clinic.ub.es
Phone +34934515950
FAX +34932271764
Barcelona, Spain
www.enerca.org

Scientific committee

Richard van Wijk- Universitair Medisch Centrum, Utrecht, the Netherlands
Eduard van Beers- Universitair Medisch Centrum, Utrecht, the Netherlands
Béatrice Gulbis- Hôpital ERASME Université Libre de Bruxelles
Joan Lluis Vives Corrons- Hospital Clinic i Provincial. Barcelona
María del Mar Mañú Péreira- Hospital Clinic de Barcelona
Patricia Aguilar-Martínez -Centre Hospitalier Universitaire de Montpellier
Michael Angastiniotis - Thalassaemia International Federation
Androulla Eleftheriou -Thalassaemia International Federation
Pilar Nicolás- Universidad de Deusto
Carlos Romeo- Universidad de Deusto

Support by an unrestricted educational grant from:

Platinum sponsor

Alexion

Gold sponsor

Addmedica

Silver sponsor

Sebia

Standard sponsor

Novartis