

INVITATION

European Haemophilia Consortium Round Table of Stakeholders on

Product related risk for inhibitor formation in Haemophilia A

Monday, March 2nd, 2015 – 13:30-17:30 (lunch included)

Royal Windsor Hotel

Brussels, Belgium

The European Haemophilia Consortium (EHC) will hold its 24th Round Table on 'Product related risk for inhibitor formation in Haemophilia A' on Monday 2 March 2015 from 13.30 till 17.30 hrs.

Haemophilia A is a rare bleeding disorder that occurs in an estimated one in 10.000 people. It is a genetic disorder linked to the X-chromosome and it affects primarily men, although in rarer cases women too. In people with haemophilia (PWH), blood does not clot normally due to a lack of specific proteins in the blood and this results in bleeds in several parts of the body. Bleeds that occur in joints and the brain can be debilitating and even fatal. To live a normal life, PWH need to replace the missing proteins by infusing coagulation factors on a regular basis. Unfortunately, an approximate 30 per cent of PWH receiving replacement factor will develop an inhibitor, an antibody to the treatment received. This means that patients will not be able to use normal treatment and will be at greater risk of disability and death. The only available current treatment for inhibitors is Immune Tolerance Induction (ITI), a very intense treatment that is extremely pricey and that often cannot be afforded by resource-limited countries. The formation of inhibitors remains one of the most challenging areas of study in haemophilia to this day, mainly because the mechanism through which inhibitors develop is still not understood.

As haemophilia is a rare disorder, it is important to study the disease on a larger population so that the disease and response to the treatment can be better understood: this is why European collaboration is so important. This round table will aim at presenting the results of several ongoing studies trying to identify the determinants for inhibitor formation in previously untreated patients (PUPs).

Prof Michael Makris, member of the EHC Medical Advisory Group, will welcome participants and introduce the event, which will start with a series of presentations reviewing current findings on factor-related risk for inhibitor development in PUPs with haemophilia A. **Prof Pier-Mannuccio Mannucci** from the University of Milan (Italy) will open presentations by discussing the results from the Survey of Inhibitors in Plasma-Products Exposed Toddlers (SIPPET) on **plasma-derived coagulation factor concentrate versus recombinant factor concentrates in PUPs**. **Prof Jenny Goudemand** from the University of Lille (France) will present **the differences between 2nd versus 3rd generation coagulation FVIII and inhibitors in PUPs**, as explored by the Research Of Determinants of INhibitor Development (RODIN) among PUPs with haemophilia, the French



Network, FranceCoag and the United Kingdom's Haemophilia Centre Doctors' Organisation (UKHCDO) research. **Dr Kathelijn Fischer** from the University of Utrecht (the Netherlands) will review **the results gathered from the reporting of inhibitors' development in PUPs into EUHASS**, a pharmacovigilance program to monitor the safety of treatments for people with inherited bleeding disorders in Europe. **Dr Alfonso Iorio** from McMaster University (Canada) will round up the topic by providing a **critical appraisal the data on inhibitor formation in PUPs according to FVIII concentrate treatment**.

The presentations will continue with **Prof Frits Rosendaal** from the University of Leiden (the Netherlands). He will give **an epidemiologist's view of the risk of inhibitors by product and how this should be assessed in future**. Finally, **Mr Thomas Sannié** from the Association Française des Hémophiles (AFH – the French Haemophilia Association) will deliver a presentation on **the role and importance of patients' reporting of adverse events and follow-up**. The floor will then be opened for discussions.

We would be delighted to have you join the conversation on March 2nd. A detailed agenda is attached. If you wish to register for this event, please **confirm your attendance by 22 February at the latest** to: laura.savini@ehc.eu.

We look forward to seeing you there,

European Haemophilia Consortium