ERN | EuroBloodNet

ERN-EuroBloodNet and ENROL Registry:

Boosting data driven research and innovative therapies & fostering best practices and access to highly specialized care

Thursday 13th June 2024 – 13:00-16:00 (CEST)

EHA Congress,
North Convention Center, Session Hall N106
Level 1, at IFEMA Madrid recinto Ferial (Fairgrounds).

European Reference Network
for rare or low prevalence complex diseases
Network Hematological Diseases (ERN EuroBloodNet)

Coordination team:

Pierre Fenaux – ERN Coordinator & Chair of oncological hub
Béatrice Gulbis – ERN Co-coordinator & Chair of non-oncological hub
María del Mar Mañú Pereira – ERN Scientific Director & ENROL Coordinator
Victoria Gutiérrez Valle – Scientific Manager
Mariangela Pellegrini – Educational & Patients Program Manager
Maria Rodríguez Sánchez – Dissemination Manager
Claire Diot – ERN Project Manager

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Agenda

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Session 1: Data driven research & Innovative therapies

Chaired by Béatrice Gulbis *(CUB-ERASME Hospital)* ***and Maria del Mar Mañú Pereira*** *(Vall d'Hebron University Hospital / Vall d'Hebron Research Institute)*

13:00 13:40 **ENROL & Data driven research & EMA Qualification – Showcases**
Béatrice Gulbis *(CUB-ERASME Hospital)*

Sickle Cell Disease: RADeep & GenoMed4All & SYNTHEMA & ERDERA
María del Mar Mañú Pereira *(Vall d'Hebron University Hospital / Vall d'Hebron Research Institute)* ***Raffaella Colombatti*** *(AO Padua)*

Acute Myeloid Leukemia: ENROL & SYNTHEMA & Impact-AML
Matteo della Porta *(IRCCS Clinical Institute Humanitas – Rozzano)*
Chiara Zingaretti *(Istituto Scientifico Romagnolo per lo Studio e la Cura dei Tumori, IRST)*

Thrombotic thrombocytopenic purpura: ENROL & T4RD
Flora Peyvandi *(Foundation IRCCS CA'Granda Ospedale Maggiore polyclinic – Milan)*

13:40 13:55 **Questions**

13:55 14:20 **ERN-EuroBloodNet sponsoring Clinical Trials and promoting innovative therapies**
María del Mar Mañú Pereira *(Vall d'Hebron University Hospital / Vall d'Hebron Research Institute)*

Mitapivat in hereditary anemias (SATISFY)
Andreas Glenthoj *(Rigshospitalet)*

New methodological approaches on Clinical Trials in Rare Diseases
Ralf-Dieter Hilgers *(MTZ - Medizintechnisches Zentrum)*

14:20 14:30 **Questions**

14:30 14:45 **Break in the room**

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Session 2: Clinical Practice Guidelines & Access to Highly Specialized Care

Chaired by Luca Malcovati *(Foundation IRCCS Polyclinic San Matteo)* ***Noemi Roy*** *(Oxford University Hospitals NHS Foundation Trust)*

14:45 15:15 **ERN-EuroBloodNet New CPGs/CDSTs produced by the ERN**
Luca Malcovati *(Foundation IRCCS Polyclinic San Matteo)*

CPG on Diagnosis and treatment of Burkitt Lymphoma in adults
Vincent Ribrag *(Gustave Roussy Hospital)*

Recommendations on Sickle Cell Disease
Béatrice Gulbis *(CUB-ERASME Hospital)*

Creating patient journeys from Patient Therapeutic Educational Program - Pilot of Von Willebrand Disease
Mariangela Pellegrini *(APHP – Saint Louis)*

15:15 15:40 **EU Mappings for the availability of Highly Specialized Procedures and Access to Treatments -** ***Victoria Gutierrez Valle*** *(Vall d'Hebron University Hospital / Vall d'Hebron Research Institute)*

Management of Venous Thrombosis and Arterial Thrombosis in Pediatric patients
Rubén Berrueco *(Hospital de Sant Joan de Déu- Hospital de la Santa Creu i Sant Pau)*
Access to Treatments: Paroxysmal nocturnal hemoglobinuria ***Pascale Burmester*** *(Secretary of the PNH Global Alliance)*

15:40 16:00 ***Questions & Closure***

Closing

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Legend:

CPGs/CDST: Clinical Practice Guidelines and Clinical Decision Supporting Tools

CPMS: Clinical Patient Management System

EDIT-SCD: Assessing efficacy and safety of genome EDITing approaches for Sickle Cell Disease

EMA: European Medicine Agency

ENROL: European Rare Blood Disorders Platform

ERDERA: European Rare Diseases Research Alliance

GenoMed4All: Genomics and Personalized Medicine for all through Artificial Intelligence in Haematological Diseases

IMPACT-AML: Master framework and pragmatic clinical trial for relapse or refractory acute myeloid leukemia

RADeep: Rare Anaemia Disorders European Epidemiological Platform

Satisfy: Safety and Efficacy of Mitapivat in Adult Patients with Erythrocyte Membranopathies

SYNTHEMA: Synthetic generation of hematological data over federated computing frameworks

T4RD: Together for Rare Diseases

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