



## **ERN-EuroBloodNet and ENROL Registry:**

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

Thursday 13<sup>th</sup> June 2024 – 13:00-16:00 (CEST)

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



### **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

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



## Session 1: Data driven research & Innovative therapies

*Chaired by **Béatrice Gulbis** (CUB-ERASME Hospital) and **María 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**

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**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 pathways 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 Berrueto** (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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