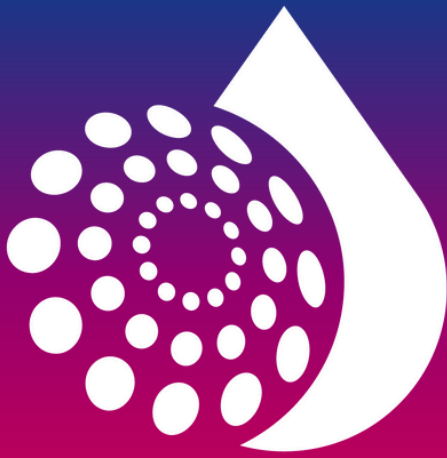


21st November 2024



ERN | EuroBloodNet

6th Progress Meeting

Present the current status and new actions over the last year

Coordination team:



Pierre Fenaux – Coordinator & Chair of oncological hub



Béatrice Gulbis – Co-coordinator & Chair of non-oncological hub



María del Mar Mañú Pereira – Scientific Director & ENROL Coordinator



Victoria Gutiérrez Valle – Scientific manager



Giulio Sannasardo – ERN Project manager



Claire Diot – ERN Operations Manager



Daiana Lopez – Dissemination Manager



Mariangela Pellegrini – Educational & Patients Program Manager



Carles Garcia Linares – ENROL Project Manager



Sara Reidel – ENROL Biostatistician & Data manager



Invited speakers (in order of presentation):



Luca Sangiorgi - ERN BOND Coordinator, Istituto Ortopedico Rizzoli



Till Voigtländer - JARDIN coordinator, Universitätsklinikum - AKH Wien



Ana Rath - ORPHANET Director, ERDERA pillar leader, INSERM



Luca Malcovati - Foundation IRCCS Polyclinic San Matteo



Anna Vanderfaeillie - CHU Saint-Pierre



Rubén Berrueco - Hospital de Sant Joan de Déu- Hospital de la Santa Creu i Sant Pau



Sarah de Vlieger - European Hematology Association



Annarita Miccio - Institut Imagine



Baiba Ziemele - ePAG, European Haemophilia Consortium (EHC)



Loris Brunetta - ePAG, Thalassaemia International Federation



Maria Pigin - ePAG, PNH Support and PNH Global Alliance



Franz Schaefer - ERKNet coordinator, Heidelberg University



Tarek El-Galaly - Aarhus University Hospital



Jan Cornelissen - ERASMUS MC



Pierre Aumont - ePAG, ELLyE & CLLAN



Étienne Lengline - APHP - Hopital Saint-Louis



Werner Zinkand - MPN Advocates Network



Andreas Glenthøj - Rigshospitalet



Ralf-Dieter Hilgers - MTZ - Medizintechnisches Zentrum

MEETING AGENDA

DATE
21/11/2024

TIME
9 AM - 5 PM

LOCATION
ONLINE

Opening plenary session

09:00 09:10 **Welcome & Agenda** – **Pierre Fenaux** (AP-HP – Saint Louis) & **Béatrice Gulbis** (H.U.B. Hôpital Erasme)

09:10 10:10 **ERNs & Joint Partnerships** – **Chair: Maria del Mar Mañú Pereira** (Vall d'Hebron University Hospital)

- **ERNs State of play** - **Luca Sangiorgi** (ERN BOND, ERNs CG coordinator, Istituto Ortopedico Rizzoli)
- **Rare Disease Joint Action: JARDIN** – **Till Voigtländer** (JARDIN Coordinator, Universitätsklinikum - AKH Wien)
- **Data services for RD research: from the EJP RD Virtual Platform to ERDERA Data Service Hub** - **Ana Rath** (ORPHANET, ERDERA Pillar leader, INSERM)

10:10 10:30 **Questions & Discussion**

10:30 10:45 **Break**

Clinical Practice Guidelines & Access to Highly Specialized Care

Chair: Luca Malcovati (Foundation IRCCS Polyclinic San Matteo) & **Victoria Gutierrez Valle** (Vall d'Hebron University Hospital)

10:45 11:15 **ERN-EuroBloodNet New CPGs/CDSTs produced by the ERN** - **Luca Malcovati** (Foundation IRCCS Polyclinic San Matteo)

- **CPGs/CDSTs on oncological diseases** – **Pierre Fenaux** (AP-HP – Saint Louis)
- **CPGs/CDSTs on non-oncological diseases** – **Béatrice Gulbis** (H.U.B. Hôpital Erasme)
- **CDSTs for patients** – **Mariangela Pellegrini** (APHP – Saint Louis)

11:15 11:35 EU Mappings to improve rare hematological diseases patients' care – Victoria Gutierrez Valle (Vall d'Hebron University Hospital)

- **Phlebotomy on Sickle Cell Disease** – Anna Vanderfaillie (CHU Saint-Pierre)
- **Arterial Thrombosis and Venous Thrombosis in Pediatric patients** – Rubén Berruenco (Hospital de Sant Joan de Déu- Hospital de la Santa Creu i Sant Pau)

11:35 11:50 Questions & Discussion

Education

Chair: Pierre Fenaux (APHP – Saint Louis) & Mariangela Pellegrini (APHP – Saint Louis).

11:50 12:00 Educational actions

- **Overview for ERN-EuroBloodNet actions for health professionals and patients** - Mariangela Pellegrini (APHP – Saint Louis)
- **EHA&ERN-EuroBloodNet Spotlight on Rare Hematological Diseases** - Sarah de Vlieger (European Hematology Association)

12:00 12:20 Collaborative programs for education on research

- **GenoMed4All & SYNHEMA & ERN-EuroBloodNet Educational program on Artificial Intelligence in hematology** – Maria del Mar Mañú Pereira (Vall d'Hebron University Hospital) & Mariangela Pellegrini (APHP – Saint Louis)
- **EDIT-SCD – Patient e-workshop on Gene editing** - Annarita Miccio (Institut imagine)
- **Educational program for patients advocates: Haemoglobinopathies in European Liaison of Medicine and Science (HELIOS)** - Mariangela Pellegrini (APHP – Saint Louis)

12:20 12:35 Questions & Discussion

CPMS & Cross Border Health

Chair: **Béatrice Gulbis** (H.U.B. Hôpital Erasme) & **Mariangela Pellegrini** (APHP – Saint Louis).

12:35 12:45 CPMS 2.0 - Béatrice Gulbis (H.U.B. Hôpital Erasme)

12:45 12:55 Repository of experts & Disease cards - Victoria Gutierrez Valle (Vall d'Hebron University Hospital)

12:55 13:05 Assistance to patients: Infopoint expectations from patients perspective - Baiba Ziemele (ePAG, European Haemophilia Consortium), **Loris Brunetta** (ePAG, Thalassaemia International Federation), **Maria Piggini** (ePAG, PNH Support and PNH Global Alliance).

13:05 13:15 Questions & Discussion

13:15 14:00 Lunch

Data Driven research & Innovative therapies

Chairs: **Pierre Fenaux** (APHP – Saint Louis) & **Maria del Mar Mañú Pereira** (Vall d'Hebron University Hospital).

14:00 15:15 The future of data sharing in EU

- **ERDERA Clinical Research Network WP9&WP10 - Franz Schaefer** (ERKNet coordinator, Heidelberg University) & **Maria del Mar Mañú Pereira** (Vall d'Hebron University Hospital).
- **Real World Evidence in the evaluation of new drugs**
 - **Presentation of results of EMA-EHA survey on RWE in regulatory and clinical decisions - Tarec El-Galaly** (Aarhus University Hospital)
 - **Building quality sources for RWE: the HOVON and Dutch Cancer registry experience - Jan Cornelissen** (ERASMUS MC)
 - **Patients perspective - Loris Brunetta** (ePAG, Thalassaemia International Federation) & **Pierre Aumont** (ePAG, ELLyE & CLLAN).

- **ENROL contribution to Epidemiology surveillance & Data driven research**
 - **ENROL Registry Module for the Collection of annual counts of RHDs patients (EpiBlood)** – Sara Reidel (Vall d’Hebron University Hospital).
 - **ENROL supporting RHD RWD standardization to advance research collaboration** – Maria del Mar Mañú Pereira (Vall d’Hebron University Hospital).

15:15 15:40 Questions & Discussion

15:40 16:10 Fair pricing of new medicines in hematology

- **Clinicians perspective - Étienne Lengline** (APHP – Hopital Saint-Louis)
- **Patients perspective: the case on PNH** – Maria Piggini (PNH Support and PNH Global Alliance).
- **Patients perspective: the example of interferons in MPN** – Werner Zinkand (MPN Advocates Network)

16:10 16:30 ERN-EuroBloodNet sponsoring Clinical Trials and promoting innovative therapies – Pierre Fenaux (APHP – Saint Louis)

- **Mitapivat in hereditary anemias (SATISFY)** - Andreas Glenthøj (Rigshospitalet)
- **Luspatercept in rare inherited anemias (LUSPARA)** – Pierre Fenaux (APHP – Saint Louis)
- **Realized: New methodological approaches on Clinical Trials in Rare Diseases** - Ralf-Dieter Hilgers (MTZ - Medizintechnisches Zentrum)

16:30 16:45 Questions & Discussion

16:45 17:00 Wrap up & Closure

Closing

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

ELLYE & CLLAN: Ensemble Leucémie Lymphomes Espoir & Chronic Lymphocytic Leukemia

Advocates Network

EHA: European Hematology Association

EMA: European Medicine Agency

ENROL: European Rare Blood Disorders Platform

ePAG: European Patients Advocacy Group

ERDERA: European Rare Diseases Research Alliance

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

Haematological Diseases

MPN: Myeloproliferative Neoplasms

PNH: Paroxysmal Nocturnal Hemoglobinuria

SYNTHEMA: Synthetic generation of hematological data over federated computing frameworks