ERN | EuroBloodNet
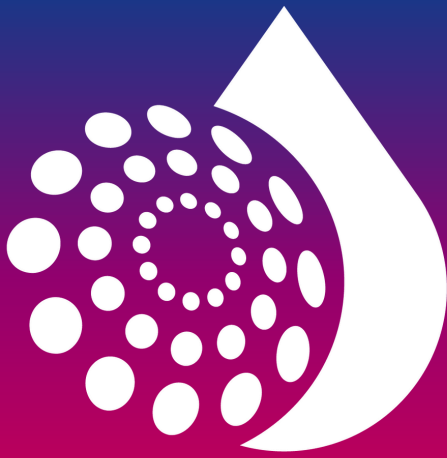
6th Progress Meeting

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

Serge Znamensky – CPMS & Educational Manager for health professionals

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 Piggin - ePAG, PNH Support and PNH Global Alliance

Franz Schaefer - ERKNet coordinator, Heidelberg University

Tarec 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 Glenthoj - Rigshospitalet

Ralf-Dieter Hilgers - MTZ - Medizintechnisches Zentrum

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 – LHUB-ULB)

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)
- **ERNs role in the new European Rare Diseases Research Alliance (ERDERA) -** 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 – LHUB-ULB)
- **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 Vanderfaeillie (CHU Saint-Pierre)
- **Arterial Thrombosis and Venous Thrombosis in Pediatric patients –** Rubén Berrueco (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 -** Serge Znamensky (Université Libre de Bruxelles) & 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 & SYNTHEMA & 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 – LHUB-ULB) & Mariangela Pellegrini (APHP – Saint Louis).

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

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 Piggin (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**
  - o **ENROL Registry Module for the Collection of annual counts of RHDs patients (EpiBlood) –** Sara Reidel (Vall d'Hebron University Hospital).
  - o **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 Piggin (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 Glenthoj (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