ERN-EuroBloodNet 5th Progress Meeting
9th November 2023

European
Reference
Network
for rare or low prevalence
complex diseases

✿ **Network**
Hematological
Diseases (ERN EuroBloodNet)

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 Registry coordinator
Victoria Gutiérrez Valle – Scientific manager
Giulio Sannasardo – ERN Project manager
Adeline Gladieux – Financial and Administrative Officer
Mariangela Pellegrini – Educational & Patients Program Manager
Maryam Lalem - Educational Activities for Health Professionals Manager
Maria Rodríguez Sánchez – Dissemination Manager
Claire Diot – ENROL Registry manager
Sara Reidel – ERN Bio-Statistician

Co-funded by
the European Union

9:00 – 10:45 Opening Plenary Session

09:00	09:30	**Welcome-** *Pierre Fenaux, Béatrice Gulbis, María del Mar Mañú Pereira*
09:30	10:00	**ERNs Strategic vision: looking back and forward -** *Holm Graessner (ERN RND)*
10:00	10:30	**Rare Disease Partnership: ERDERA -** *Yanis Mimouni*
10:30	10:45	**Discussion**

10:45 – 11:00 Coffee Break

11:00 – 13:00 ERN-EuroBloodNet Strategic vision: Looking back and forward

Looking back: Plenary session presenting ERN-EuroBloodNet concrete actions and successes

11:00	11:30	**Best Practices**

- Repository of CPGs/CDSTs & New CPG/CDST produced by the ERN - *Luca Malcovati 10'*
  - Use case: CPG on Sickle Cell Disease – *Béatrice Gulbis 5'*
- EU Mappings for the availability of Highly Specialized Procedures - *Victoria Gutierrez Valle 5'*
  - Use case: Management of Venous Thrombosis and Arterial Thrombosis in Pediatric patients - *Heleen van Ommen / Ruben Berrueco 5'*
- Creating patient Journeys from Patient Therapeutic Educational Program. - *Mariangela Pellegrini 5'*

11:30	12:10	**Education**

- For Health Professionals:
  - Webinars programs & Preceptorships – *Maryam Lalem 7'*
  - eLearning platform – *Beatrice Gulbis 5'*
- For Patients:
  - Trainings & New program on transition – *Mariangela Pellegrini 7'*
- Collaborative programs for education on research:
  - ENROL webinars program & Knowledge pills for patients – *Loris Brunetta / Maria Piggin 5'*
  - Genomed4ALL Educational program on Artificial Intelligence in hematology for public-at-large – *Maria del Mar Mañú Pereira 5'*
  - EDIT-SCD – *Mario Amendola 5'*
  - Haemoglobinopathies in European Liaison of Medicine and Science (HELIOS)- *Petros Kountouris 5'*

12:10	12:30	**Cross Border Health & CPMS**

- Cross border health & CPMS – *Béatrice Gulbis 7'*
- Repository of experts and Search tool & Disease cards– *Maria Rodriguez 5'*
- Assistance to patients: Infopoint & Collaboration with Ukranian hub – *Mariangela Pellegrini 7'*

Looking forward: what would you like to see?

12:30   13:10   Discussion
- Can any action be replicated in your area? Would you be willing to lead it?
- Do you identify a different type of action that still has not been tackled by the ERN? Would you be willing to lead it?
- From topics presented, what are the most interesting topics to be further explored in the frame of the ERN?

13:10   13:15   Conclusions from discussion *Pierre Fenaux and Béatrice Gulbis*

13:15 – 14:00 Lunch Break

14:00 – 16:40 ERN-EuroBloodNet advancing on clinical research

Regulatory aspects of rare disease patient's registries: Pathway to cooperation with EMA. Moderators : María del Mar Mañú Pereira and Matteo della Porta

14:00   14:30   Certification of ERNs registries by the EMA for regulatory and decision making porpoises – *Carla Jonker (EMA)*

14:30   15:30   How can ERNs registries improve the Quality of Data for EMA validation and re-use? ENROL Use case - *María del Mar Mañú Pereira 15'*
- Validation of synthetic data: GenoMed4All and Synthema – *Matteo della Porta 15'*
- Re-use in Clinical trials: IMPACT-AML – *Giovanni Martinelli 15'*
- PROMs in reimbursement decision making process -RD Catalyst grant – *Vicky McGrath 15'*

15:30   15:45   Proposal for new Directive on medicinal products for human use: how will impact us? – *Simone Boselli (EURORDIS public affairs) 15'*

15:45   16:00   Discussion

ERN-EuroBloodNet Improving access to diagnosis and treatments. Moderator : Pierre Fenaux

16:00   16:10   ERN-EuroBloodNet sponsoring clinical trial - *Pierre Fenaux 3'*
- Use case: SATISFY **-** *Andreas Glenthoj / Eduard van Beer 7"*

16:10   16:25   EU mappings on access to treatments
- Use case: Paroxysmal nocturnal hemoglobinuria – *Maria Piggin 7'*
- Use case: Myeloproliferative neoplasms disorders - *Jean-Jacques Kiladjian / Werner Zinkand 7'*

16:25   16:50   Discussion

16:50 – 17:00 Closing

16:50   16:55   **ERN-EuroBloodNet New video! -** *Maria Rodriguez*

16:55   17:00   **Wrap-up and closing -** *Pierre Fenaux, Béatrice Gulbis, María del Mar Mañú Pereira*

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

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

SYNTHEMA: Synthetic generation of hematological data over federated computing frameworks