ERN-EuroBloodNet meeting at the European Hematology Association Congress 2023

The Role of ERN-EuroBloodNet and ENROL Registry on Advancing Translational and Clinical Research in Hematology

8th June 2023 Frankfurt

ERN | EuroBloodNet

European Reference Network | Hematological Diseases (ERN EuroBloodNet)

Co-funded by the European Union

Co-funded by the European Union.
Views and opinions expressed are however those of the author(s) only and do not necessarily reflect those of the European Union or European Health and Digital Executive Agency (HaDEA). Neither the European Union nor the granting authority can be held responsible for them.

ERN-EuroBloodNet meeting "The Role of ERN-EuroBloodNet and ENROL Registry on Advancing Translational and Clinical Research" was held at the European Hematology Association Congress last 8th June 2023 in Frankfurt.

70 participants from 14 EU Member States attended the meeting. Remarkably 60% of participants were Members representatives or part of the multidisciplinary teams, while 7% were ePAGs and patients representatives (Fig 1). The most represented areas of expertise were Red blood cell disorders (27%), Myeloid subnetwork (22%) and Bone marrow failures (21%) (Fig 2).

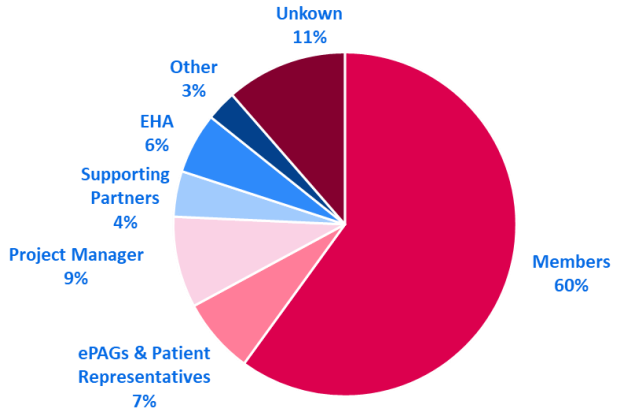

Fig 1. Role of participants of the meeting

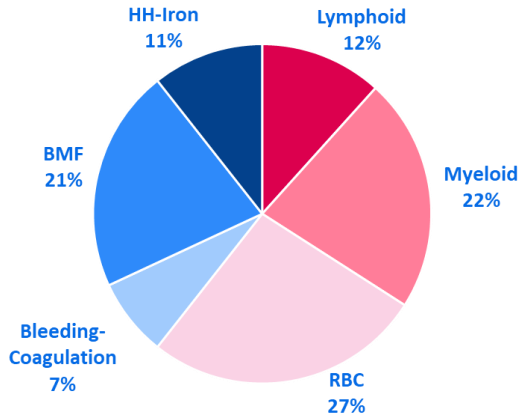

Fig 2. Area of expertise of participants. RBC: Red blood cell disorders, BMF: Bone marrow failures, HH-Iron: Haemochromatosis and iron related disorders

Session 1: Improving access to diagnosis and treatments

Conducting clinical trials and research is getting more and more complex and the EU is slowly falling behind other economic areas, especially for rare diseases. Is it bureaucracy, lack of collaboration, or something else? Session 1 focussed on the ERN-EuroBloodNet ongoing actions to assess the current access to highly specialized diagnostic procedures and treatments as the basis for the identification of key barriers and steps to improve their access:

- The European Rare dIsease research Coordination and support Action (ERICA) project was introduced, which aims to establish a structural framework in support of the research activities of the ERNs, including to improve the progress of clinical trials in EU. The joint efforts from the 24 ERNs to push challenges and barriers on the agenda of other stakeholders including payers, pharma regulators and EMA can really make a difference in the development of rare disease policies.
- The SATISFY study (Mitapivat in red blood cell membranopathies) is the first ERN-EuroBloodNet sponsored clinical trial. The model can be used as a reference for the development of new clinical trials in other rare haematological diseases. It includes the use of validated PROMs, and it is endorsed as one of the pilots conducted at ERICA WP3 on Patient centered research for implementation of patient self-reported outcomes in clinical research.

- Paroxysmal nocturnal hemoglobinuria, myeloproliferative disorders and thrombotic thrombocytopenic purpura are rare haematological diseases with the shared gap of the heterogeneity of access to diagnosis and/or treatments in all the EU Member States. ERN-EuroBloodNet together with health professionals and patients are conducting EU mappings to:
  - ✓ Assess the availability and accessibility to highly specialized diagnosis and orphan drugs
  - ✓ Lobbying to obtain access to diagnosis procedures and orphan drugs in EU Member States
  - ✓ Improving the visibility of Orphan Drugs to the general public and policymakers
  - ✓ Strengthen the collaboration between patients and physicians

"

Suggestions from the audience included:

- ✓ Conduction of a new mapping exercise on access to treatments for myelodysplastic syndromes
- ✓ Integration of the mapping exercises within the ENROL registry in order to systematically put together access to diagnosis and treatments and number of patients by disorder and Member States
- ✓ Needs of understanding how to analyse results in terms of the role of National Health bodies, the pricing of the drug and their negotiations at national market level.

"

Session 2: ENROL Registry and endorsed initiatives

Session 2 was focussed on the European Rare Blood Disorders Platform (ENROL) as the ERN-EuroBloodNet central registry for the promotion of research and epidemiological surveillance of rare haematological diseases. ENROL has developed a strategy to connect and facilitate the upgrade of existing rare haematological diseases registries in EU while promoting the building of new ones when / where lacking.

"

Following this strategy, ENROL managed to:

- ✓ Increase the interoperability with the already existing RADeep registry, the Rare Anaemia Disorders European Epidemiological Platform, allowing the collaboration for data sharing with 12 EU Member States and Norway.
- ✓ Promote the creation of 2 new sub-registries in the following areas:
  - EU-Blast: Blastic Plasmacytoid dendritic cell neoplasm
  - TWIST: Bleeding and coagulation disorders: von willebrand disease
- ✓ Lead data re-use strategy and data standardization in EU-funded research projects
  - GenoMed4All: Sickle cell disease and myelodysplastic syndromes
  - Synthema: Sickle cell disease and acute myeloid leukemia
  - IMPACT-AML: Acute myeloid leukemia

"

Session 3: Artificial Intelligence (AI) based models in hematology: Genomed4all and Synthema

ENROL has positioned itself as a key player in Europe for strengthening the use and re-use of health data on rare haematological diseases for the provision of best healthcare and research.
Session 3 focussed on the collaborations with following EU funded projects linked to the re-use of patients data:

- Genomics and Personalized Medicine for all though Artificial Intelligence in Haematological Diseases (GenoMed4All GA101017549) and Synthetic haematological data over federated computing frameworks (Synthema GA101095530) supports the pooling of genomic, clinical data and other "-omics" health through a secure and privacy respectful data sharing platform based on the novel Federated Learning scheme, to advance research in personalised medicine in haematological diseases thanks to Artificial Intelligence models and standardized interoperable sharing of cross-border data, without needing to directly share any sensitive clinical patients' data.
- Use cases on myelodysplastic syndromes, acute myeloid leukemia and sickle cell disease are led by ERN-EuroBloodNet members. GenoMed4All & Synthema will start by leveraging on ERN-EuroBloodNet healthcare information and repositories that will be standardized through ENROL and RADeep Registries.

> The use of synthetic data was highlighted as the future of medicine by the development of scalable technologies applicable to other diseases.
>
> ✓ The data quality is ensured at the primary data collection by promoting the standardization and codification of data and ensure statistical properties checks

In conclusion, networking, synergies between different ERN stakeholders, as well as registries were presented during the meeting; they determine new ways of working together for years to come while ensuring long-term sustainability.

Thank you all for your active participation!
Contribute to this brief satisfaction survey! Your feedback is really valuable for us!

https://forms.gle/YZpbkW9Q4A9SzS2A6

Presentations are available on the European Collaborative Platform and on demand by contacting

Maria.rodriguez.sanchez@vhir.org