

3rd ERN-EuroBloodNet

Board of the Network meeting

Main outcomes

13th and 14th November 2019, Barcelona

Vall d'Hebron University Hospital

Conference Hall - Teaching Pavilion UAB
(Sala d'Actes – Pavelló Docent UAB)



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
Victoria Gutiérrez Valle – IT and dissemination manager
Mariangela Pellegrini – ERN manager



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The 3rd EuroBloodNet Board of Network meeting was held last 13 and 14 November in Vall d'Hebron University Hospital, Barcelona, with the participation of more than 80 attendants. The audience included member representatives, patients' organizations, candidates for new members, affiliated partners and collaborating stakeholders as the European Hematology Association (EHA), the European School of Hematology (ESH) or International Quality Expertise (UKNEQAS).

In addition to plenary sessions, on this occasion the meeting counted with two Parallel Sessions where the main actions and results implemented by the network were presented by the projects coordinators finalizing with key questions to be discussed by the audience divided into areas of expertise.

Finally, an overview of the ERNs and members monitoring system including the set of indicators defined by the EC and ERNs WG on monitoring was provided by Victoria Gutierrez Valle, and an update on the ERNs WG on Research was given to the participants thanks to the attendance of Eduard van Beers to the meeting ERNs Coordinators Group meeting in Brussels, overlapping on the dates with the EuroBloodNet meeting.

Plenary Session and Round table on European Reference Networks (ERNs) Consolidation & Sustainability

The meeting started with a Plenary Session and Round table on European Reference Networks (ERNs) Consolidation & Sustainability. The Plenary included an overview of the impact of ERNs at hospital level and national health systems by Yolima Cossio (Director of Information Systems in Vall d'hebron Barcelona Hospital Campus), the ERNs consolidation and long term sustainability by Enrique Terol (DG SANTE – European Commission) and the European Joint Programme (EJP) on Rare diseases and its links to ERNs by Claudio Carta (EJP WP 14 leader).

Since ERNs are not research projects but Institutional networks consensus was reached on the paramount role of CEOs for supporting the four pillars for ERNs consolidation: Governance, geographical coverage and access, budget (cost analysis) and outcomes.

The integration of the ERNs in the National healthcare systems was also highlighted as one of the main challenges and priorities for ERNs long-sustainability

Parallel Session I: Cross border health, Interprofessional consultations and Webinars program

Block A: European mapping of expertise in RHD

Drivers: R Colombatti, B de la Salle, P Fenaux, S Román, MM Mañú Pereira

EuroBloodNet new experts' profiles and disease grouping: relevance for cross border health and interaction with stakeholders - MM Mañú Pereira

EuroBloodNet mapping of healthcare services in Rare Hematological Diseases (RHDs) and its monitoring based on key performance indicators (KPIs) is cornerstone for setting the basis for a European model of networking. They rely on a validated identification of experts and Highly specialized procedures (HSP) at both the national and European levels. It also arises the need for cross border health based on existing gaps in certain Member State for clinical management of a specific condition while allows health planning and better allocation of resources.

Making this expertise public through EuroBloodNet members' profile allows the establishment of **new bridges for collaboration** not only among experts but also with other European bodies such as EMA while enabling health professionals and patients seeking for best healthcare services across Europe. The **EuroBloodNet mapping of healthcare services** in RHD will also provide valuable information for identifying needs on best practices, continuing medical education and clinical and translational research. In addition, the **incorporation of KPIs** on patients' activity and HSPs will also contribute to the EC assessment of network and members' excellence.

Through this activity EuroBloodNet aims to build a **central repository of reliable sources** of information on expertise available at both national and European level in RHDs. To this aim, RHDs have been grouped into disease or disease groups (RHD-DGs) based on the analysis of codification schemes (ORPHA and ICD) for definition of rare diseases (RDs). **Disparities and gaps identified** will be reported to corresponding bodies.

RHD-DGs will be used for a) the members' reporting of number of patients and new patients, b) make the expertise in EuroBloodNet searchable and c) classify the contents of the website, i.e. Guidelines, educational material.

Pilot case for cross border Bone marrow transplant on non-oncological diseases among two EuroBloodNet members – R Colombatti

Given the scarcity and heterogeneous distribution of expertise on certain RHDs, it may be common for patients that the most appropriate care is offered in another MS. Bone Marrow Transplantation (BMT) is a procedure widely implemented and standardized for oncological RHDs, however expertise and capacity required for its performance on non-oncological RHDs differ from the medical perspective and present high inequalities on its availability across EU. Accordingly, EuroBloodNet identified BMT for non-oncological diseases as an HSP priority for an action on the field of cross border health.

An online survey was conducted among the 56 EuroBloodNet HCPs covering non-oncological diseases for the analysis of BMT need and availability, while a pilot cross border agreement for BMT on Sickle Cell Disease is being established among a member from Italy and a member from Ireland.

External Quality Assessment for rare haematological diseases – B de la Salle

Pyruvate kinase deficiency (PKD) is the first most common inherited red cell enzyme deficiency leading to chronic non-autoimmune non-spherocytic haemolytic anaemia. There is currently no external quality assessment (EQAS) for the assay of PK activity and the small number of laboratories performing the assay in each member state means that an international EQAS developed in collaboration with EuroBloodNet is required for effective performance assessment.

Check the new website section for External Quality Assessment at EuroBloodNet website: <https://www.eurobloodnet.eu/external-quality-assessment/>

Main outcomes from the Block:

- ✓ Disease groups were reviewed by experts during the parallel sessions.
- ✓ “Key” procedures Standards of Care only available in members and not always available at the national level, thus potential targets of Cross border health were revised
- ✓ Besides de on-going projects on European mapping of availability for BMT and NGS for non-oncological disorders, new projects were proposed:
 - Non-oncological: European mapping on availability of Transcranial Doppler for SCD, of T2*MRI for assessment of iron overload for beta- thalassaemia, and of gene therapy for beta – thalassaemia.
 - Oncological: European mapping on availability of CAR-T cell therapy, and of diagnostic procedures, treatment and follow-up of primary intra-ocular lymphomas
- ✓ New project: to set up an inventory of available EQAs for RHDs
- ✓ New project: Development of new External Quality Assessment for hepcidin assay and EMA Binding test

Block B: Interprofessional consultations and Webinars program

Drivers: P Aguilar-Martínez, D Bron, F Cerisoli, B Gulbis, V Gutierrez and M Pellegrini

Clinical Patient Management System (CPMS): expert panels for cross border advice – B Gulbis

The Clinical Patient Management Platform (CPMS) is a virtual platform General Data Protection Regulation (GDPR) compliant supported by the EC for ERNs inter-professional consultation of complex cases.

https://ec.europa.eu/health/sites/health/files/ern/docs/cpms_ps_en.pdf

The CPMS enables uploading patient data in a harmonized and specialized format which can be customized according to RHD-DGs needs. It allows not only uploading several formats of medical documents and images but also organizing videoconferences for interactive discussion.

Check the new website section for CPMS at EuroBloodNet website:

<https://www.eurobloodnet.eu/cpms/about-cpms/>

Webinars program for patients and health professionals – P Aguilar-Martinez and D Bron

EuroBloodNet Webinars are online educational activities conducted using the Webex Platform provided to EuroBloodNet by the European Commission. It allows full audio and visual communication, as well as interaction between webinar’s speaker and participants.

Three types of webinar program are planned for 2020:

- 1) EuroBloodNet Thursdays: the objective is promoting the interest on very innovative topics in order to stress among health professionals the cutting-edge advances in the field of Rare Hematological Diseases. Webinars are mainly addressed on complex disorders, highly specialized procedures and implementation of guidelines. Apart from defining a focus of interest that could be neglected because too specialized or dedicated to ultra-rare diseases, webinars give also visibility to upcoming educative events organized by EHA and ESH with respect to the session’s topic. Check the dedicated section at EuroBloodNet website: <https://www.eurobloodnet.eu/education/webinars/>
- 2) Webinar program on cutaneous lymphoma / pyruvate kinase deficiency: comprehensive webinar program focused on ultra-rare disorders – Accreditation
- 3) Webinar program for patients: the objective is promoting awareness on very rare hematological diseases among patients, patients relatives and caregivers. Apart from defining a focus of interest that could be neglected because dedicated to very rare diseases, webinars would also give the visibility to events organized by patients organizations with respect to the session’s topic. A pilot of a comprehensive program dedicated to Cutaneous Lymphoma will be contacted in collaboration with Europe Lymphoma Coalition.

Main outcomes from the Block:

- ✓ The importance for using CPMS was highlighted, especially regarding:
 - Compliance with GDPR
 - Making available the expertise for the non-experts, and also among experts.
 - Tracking patients within the healthcare providers.
- ✓ Major barriers identified in the use of the CPMS was the time required for enrolling the patient and steps to follow until the final outcome is achieved. It was clarified that a “shorten” flowchart is being developed by the EC to simplify the process.
- ✓ Some issues were also arisen regarding:
 - Advice for treatments: how could you give a European advice on treatment if all EU MS do not have the same access to treatment? Cross border Health action is required in this area
 - A potential improvement of the system is including Key Words on the cases to search easier for the case at the platform.
- ✓ EuroBloodNet Thursdays webinars has been launched to increase awareness on new diagnostic and therapeutic approaches on RHDs, a list of upcoming webinars were identified <https://www.eurobloodnet.eu/education/webinars/> you may suggest new topics by contacting Mariangela Pellegrini.
- ✓ CPMS and webinar programs may be complementary approaches for improve access to diagnosis and treatment for ultra-rare disorders i.e. cutaneous lymphoma, rare iron related metabolism disorders

Thursday 14th November - Parallel session II: Registries, clinical trials, best practices and patients' actions

Block A: Patients' registries and clinical trials

Drivers: F Cerisoli, B Gulbis, P Fenaux, V Gutiérrez and MM Mañú Pereira

EuroBloodNet proposal for rare haematological diseases (ENROL) - B Gulbis and MM Mañú Pereira

ENROL, the European Rare Blood Disorders Platform, has been conceived in the core of EuroBloodNet as an **umbrella for both new and already existing registries** on rare haematological disorders (RHDs). ENROL aims at **avoiding fragmentation** of data by promoting the standards for patient registries' interoperability released by the EU RD platform. Accordingly, ENROL will map at the EU level demographics, survival rates, diagnosis methods, genetic information, main clinical manifestations and treatments in order to obtain **epidemiological figures and identify trial cohorts for basic and clinical research**. To this aim, ENROL will **connect and facilitate upgrading of existing RHD registries**, while **promoting the building of new ones when / where lacking**.

Check the dedicated section at EuroBloodNet website: <https://www.eurobloodnet.eu/enrol/>

EuroBloodNet sponsoring Clinical trials – P Fenaux

In order to establish the state of the art of on-going CTs for rare hereditary anaemias (RHAs) a desk research was conducted on ClinicalTrials.gov website. 122 "Search terms" covering 105 disorders classified as rare hereditary anemias were established based on ORPHA classification. A total of 256 CTs resulted from the analysis after removing duplications and assess quality of data regarding disease focus. Only 26 RHA from the 105 disorders classified as RHA (25%) are currently covered by at least one CT. This means that for 3 out of 4 very rare RHA have no CT is available, and in many cases tis results on no therapeutic option. In addition, only 19% of the CTs are open in Europe and from this, only around the 50% are active in EuroBloodNet members from only 5 member states.

Based on the results, EuroBloodNet has therefore started to conduct academic CTs in very rare hematological diseases in which pharmaceutical companies have not planned CTs including:

- 1) Luspatercept in Congenital Dyserythropoietic Anemia type II (CDAIL) and congenital sideroblastic anaemias
- 2) Hemanext in Refractory anaemia with ringed sideroblasts

Main outcomes from the Block:

- ✓ Information on existing registries covering oncological and non-oncological RHDs was gathered from attendants as well as contact details to be included in ENROL
- ✓ New project: A joint project proposal on "An international pharmaco-epidemiological registry focused on patients with acute leukemias of ambiguous lineage (ALAL), demographic characteristics, treatment options and their effectiveness"

Block B: Best practices and Patients' actions

Drivers: L Malcovatti, S Román, M Pellegrini and A Weinman

Inventory of guidelines and assessment of implementation – L Malcovati

The aim of TFA – Best Practice is to promote best practices in prevention, diagnosis and safe clinical care across Europe by creating a comprehensive public database of reliable guidelines, ranging from prevention, diagnostic tests and treatments to the organization of patient-centred care in multidisciplinary teams and patient safety.

The database aims at serving as a platform for sharing best practices, facilitating timely, effective and efficient translation of research results into patient oriented strategies at the individual and public health levels and provide professionals, patients, and policy makers with the best and most up to date information.

The documents included in the Repository of Guidelines and Recommendations have been classified according to key domains, including scope and purpose, stakeholder involvement and methodology adopted to develop the guideline / recommendation. This database provides the frame to identify critical areas in which development of new guidelines or update of existing documents is warranted.

Check the new section on Guidelines and Recommendations at EuroBloodNet website:

<https://www.eurobloodnet.eu/guidelines-and-recommendations/>

Short stays for health professionals – M Pellegrini

In order to have an exhaustive Continuing Medical Education, some highly specialized procedures, diagnosis and clinical management of very rare diseases would require not only theoretical lessons but also practical exercises held in experts' centres. Based on the results of the EuroBloodNet Questionnaire on continuing medical education and on the feedback from subnetwork coordinators, some areas for on-site training and experts centres related have been identified.

During first year of EuroBloodNet implementation, Paroxysmal nocturnal haemoglobinuria (PNH) Aplastic Anaemia (AA) were identified as one are to be benefited from the organization of short stays due to the lack of expertise in its diagnosis, leading potentially to a worsen of prognosis on the health's patient. For the second year, Sickle Cell Disease has been identified has an area of short stays' program intervention.

Highly specialized centres that will host the preceptorships are chosen among EuroBloodNet Health Care Providers, coordinated by EuroBloodNet members and represent the excellence in RHDs field at International level.

Check the dedicated section for Preceptorships at EuroBloodNet website:

<https://www.eurobloodnet.eu/education/preceptorships/>

Patients activities – M Pellegrini, A Weinman, P Aumont

Up to now, SCD is one of the few rare anaemias that do not have, in every country in Europe, an adequate educational contribution to daily management of the disease. This is the reason why EuroBloodNet is providing a representation of SCD European community able to make manifest

patients' needs and facilitate access to care. The ERN EuroBloodNet is also promoting educational activities addressed to SCD patients in order to encourage their formation as advocate and expert patients.

For the 3rd year EuroBloodNet is presenting actions carried out for patients' activities: the establishment of the repository of educational material for patients, the establishment of an EU Network for SCD patients' organization, an educational training for SCD patients and Webinars programs for patients.

Main outcomes from the Block:

- ✓ The need for patient involvement and development of documents on complementary issues in the context of taxonomy project was highlighted
- ✓ National EuroBloodNet representatives shall be designed for establishing formal contacts with National Societies and national disease-specific networks
- ✓ Identification of out-to-date guidelines/recommendations in the repository: how to manage it?
- ✓ The achievement of EHA accreditation for the Preceptorships was recommended.
- ✓ A new Cutaneous Lymphoma Task force formed by EuroBloodNet coordination team, continuing medical education coordinators, and cutaneous lymphoma experts, EURORDIS, Lymphoma Coalition Europe, and presidents of cutaneous lymphoma patients organizations has been created for organizing webinars for expert patients and Patient Organizations.
- ✓ New project: Creation of educational material for patients in collaboration with experts. First target is Methemoglobinemia.

**4th EuroBloodNet Board of Network meeting will be held
22nd and 23rd October 2020 in Paris, SAVE THE DATE!**