

ERN-EuroBloodNet Progress Meeting Outcomes

20th May, Online



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 – Educational & Patients Program Manager

Maria Rodríguez Sánchez – ERN Manager

Léa Margot – Administrative and CPMS Manager

Last 20th of May, the [ERN-EuroBloodNet](#) Progress meeting was held online. More than 150 were present during this last edition!

The meeting started with a welcome message by the [ERN-EuroBloodNet](#) coordinators [P Fenaux](#) ([Assistance Publique – Hôpitaux de Paris, Hôpital Saint-Louis](#)) and [B Gulbis](#) ([CUB-Hôpital Erasme](#)), followed by the talk “ERN-EuroBloodNet in Numbers” provided by [V Gutiérrez Valle](#) ([ERN- IT&Dissemination manager, Hospital Universitari Vall d'Hebron](#)).

The meeting was divided in 5 different sessions each:

1. Cross border health
2. Continuing medical education
3. Best practices
4. Telemedicine
5. Clinical trials and research

1. Cross border health

The first session started with a presentation provided by [R Colombatti \(AO Padua\)](#) about the availability of Transcranial Doppler (TCD) for sickle cell disease (SCD) in European Union Member States (EU-MS). The results presented were obtained through a mapping exercise conducted among the ERN-EuroBloodNet members. The second lecture was focused on the EU mapping of Diagnostic Procedures: Primary Vitreoretinal Lymphomas (PVRL). The results of this mapping were presented by [D Malaise \(Institute Curie\)](#). The third talk of this session "Very expensive procedures: the example of gene therapies" was provided by [GL Forni \(E.O. Ospedali Galliera, Genova\)](#), [P Bartolucci \(Assistance Publique-Hôpitaux de Paris, Hôpital Henri-Mondor\)](#) and [S Benghiat \(CUB-Hôpital Erasme\)](#). Finally, the last presentation of this session was "The assistance of ERN-EuroBloodNet to patients" and was in charge of [M Pellegrini \(ERN-EuroBloodNet Educational & Patients Program Manager, Assistance Publique – Hôpitaux de Paris, Hôpital Saint-Louis\)](#).

Main Points highlighted during the discussion:

- Availability of TCD for SCD in EU-MS: Preliminary analysis of the Survey's results:
 - ✓ A better implementation of the TCD guidelines is necessary
 - ✓ A collection of educational activities is necessary to teach how to perform TCD to Haematologists and other healthcare staff.
 - ✓ The creation of a network between centers is crucial for the improvement of TCD implementation
 - ✓ Healthcare reports focusing on the need to support healthcare staff are necessary to position TCD as a standard of care in Europe.

- EU mapping of Diagnostic Procedures: Primary Vitreoretinal Lymphomas (PVRL):
 - ✓ The creation of guidelines is necessary for the improvement of the PVRL patients healthcare.

- Very expensive procedures: the example of gene therapies
 - ✓ More information about the actual clinical trials is necessary before the application of these treatments in patients. One of the crucial points is that all the trials nowadays are being performed on young patients.
 - ✓ Economical investment in health treatments is one of the limits for the implementation of innovative treatments
 - ✓ More than 84% of patients are willing to receive genetic therapies
 - ✓ Better Information has to be provided to the patients about the risks of the genetic therapies.
 - ✓ New workgroup: Creation of a [ERN-EuroBloodNet](#) workgroup focused on gene therapies

- The assistance of ERN-EuroBloodNet to patients

The center [AO Padova](#) started the procedure, led by [R Colombatti](#), to provide bone marrow transplants to Irish SCD patients in the Italian center. Finally, this action promoted the implementation of this treatment in the Irish healthcare system. This represents a good result since patients will receive the treatment in their country.

2. Continuing medical education

ERN-EuroBloodNet educational sessions for [health professionals](#) and [patients](#) were presented during the following talks: “Accredited webinars for health professionals: Topic on Focus programs results and news” provided by [M Bagot](#) ([Assistance Publique – Hôpitaux de Paris, Hôpital Saint-Louis](#)) and [P Coppo](#) ([Assistance Publique - Hôpital Saint-Antoine](#)) and “Educational programs for patients: Webinars ([Cutaneous Lymphoma](#), [Myelodysplastic syndromes](#), [Sickle cell disease](#), [Congenital dyserythropoietic anemias](#))” presented by P Aumont ([ERN-EuroBloodNet ePAG](#), SILLC), N Bolaños ([Lymphoma Coalition](#)) and J Dubow ([Connaitre et Combattre les Myelodisplasies](#)). All the produced educational material in [ERN-EuroBloodNet](#) will be available in an “ERN-EuroBloodNet eLearning platform” under development as explained by [P Aguilar-Martínez](#) ([CHU Montpellier](#)). The next talk, provided by L Brunetta ([ERN-EuroBloodNet ePAG](#), [Thalassaemia International Association](#)) and A Weinman ([EURORDIS](#)), focused on the establishment of European Networks of Patients Organizations. Finally, [M Pellegrini](#) ([ERN-EuroBloodNet Educational & Patients Program Manager](#), [Assistance Publique – Hôpitaux de Paris, Hôpital Saint-Louis](#)) together with J van der Meer (Public Health and Humanitarian Aid Consultant, Phesta, [Ecorys Agency](#)) presented to the audience the future opportunities of the experts to participate in the [Preceptorships](#) and the ERN Exchange Mobility Program.

Main Points highlighted during the discussion by talks:

- Educational programs for patients: Webinars ([Cutaneous Lymphoma](#), [Myelodysplastic syndromes](#), [Sickle cell disease](#), [Congenital dyserythropoietic anemias](#))”
- ✓ Different participants expressed different aspects that need to be considered in the organization of educational activities for patients:
 - Elder patients are not used to follow webinars.
 - The language is a barrier to arrive all patients of the EU-MS.
 - The approach to patients is very linked to the culture.
 - Healthcare structure is different depending on the country
- ✓ The experience that is going to be gathered by [ERN-EuroBloodNet](#) will guide future educational activities. They will be adapted to the needs of the community, which is diverse in culture and healthcare system.
- [Preceptorships](#) & ERN Exchange Mobility Program
The establishment of the evaluation frame of the ERN Exchange Mobility Program was slowed down because of the COVID-19 situation. The criteria will be qualitative.

3. Best practices

The third session focused on a unique presentation: "[Guidelines and other Clinical Decision Support Tools](#)". The speakers, two of the experts involved in this project, are [L Malcovati](#) (ERN-EuroBloodNet chair, [Foundation IRCCS Policlinic San Matteo, Pavia](#)) and N Roy ([European Hematology Association \(EHA\)](#) Guidelines Committee Chair, [Oxford University Hospitals NHS Foundation Trust](#)).

Main Points highlighted during the discussion:

- ✓ Last February 2020, the European Commission introduced a new project to provide technical assistance to the ERNs to develop, appraise, and implement clinical practice guidelines (CPGs) and Clinical Decision Support Tools (CDSTs). The ERNs will work to adopt a common methodology and to develop their own decision-making tools. The steps are established in a roadmap of 4 years.
- ✓ [ERN-EuroBloodNet](#) has appointed a Consortium to develop the project.
- ✓ The consortium has developed several guidelines to facilitate the production of several documents. The detailed information will be available on the [ERN-EuroBloodNet website](#).
- ✓ The call on the identification of topics for development of [Guidelines and other Clinical Decision Support Tools \(CDSTs\)](#) got almost 30 proposals. In order to avoid duplication of efforts, [ERN-EuroBloodNet](#) in collaboration with the [European Hematology Association \(EHA\)](#) performed a first prioritization process, establishing a top ten list of the topics. Following steps within the EC Roadmap include the selection of two topics, one malignant and one non-malignant, for the production of full guidelines and five topics for adaptation.
- ✓ [ERN-EuroBloodNet](#) offers support for the production of documents for patients or expert consensus statements.
- ✓ Any [ERN-EuroBloodNet](#) member who has funds or internal resources for addressing a proper systematic review of the literature in a specific field can contact the guideline [ERN-EuroBloodNet](#) team. The consortium also considers promoting evidence-based guidelines independently from the consortium and then apply for the endorsement at the end of the process.
- ✓ [ERN-EuroBloodNet](#) will be also actively involved in the creation of patient pathways in collaboration with other ERNs.

4. Telemedicine

The talks of this session focused on the implementation and use of the [Clinical Patient Management System](#) in [ERN-EuroBloodNet](#). The first one called “General overview of the [Clinical Patient Management System \(CPMS\)](#)” presented by [B Gulbis](#) ([CUB-Hôpital Erasme](#)) and [L Margot](#) ([ERN-EuroBloodNet Administrative & CPMS manager, Assistance Publique – Hôpitaux de Paris, Hôpital Saint-Louis](#)). Finally, “The experience of Cutaneous Lymphoma Virtual Board” was presented by [U Wehkamp](#) ([Universitätsklinikum Schleswig-Holstein](#)) and [M Vermeer](#) ([Leiden University Medical Center](#)).

Main Points highlighted during the discussion:

- ✓ The main beneficiaries of this system in [ERN-EuroBloodNet](#) are the patients affected by ultra-rare diseases, i.g., Cutaneous Lymphoma.
- ✓ [CPMS](#) usage is also interesting in countries with a lack of expertise due to their low number of patients.
- ✓ There is a gain of knowledge between the experts involved in [CPMS](#). They take advantage of the experience of other members of the team.
- ✓ The cutaneous lymphoma (CL) group has performed fruitful work in terms of diagnosis and also simplifying the [CPMS](#) platform.
- ✓ The next steps will be:
 - To identify the ultra-rare diseases in [ERN-EuroBloodNet](#) that will be the ideal target for the usage of CPMS
 - To make the expertise accessible to the EU-MS non-experts
- ✓ Regarding this last point, the CL leader expressed that they are studying options to make the system more inclusive.

5. Clinical trials and research

During the fifth session, an overview of the different on-going research projects in [ERN-EuroBloodNet](#) was presented to the audience: “[ERICA project: the European Rare disease research Coordination and support Action](#)” provided by AM Pereira ([ERICA Coordinator, Leiden University Medical Center](#)), “[Genomed4all project: Genomics and Personalized Medicine for all through Artificial Intelligence in Haematological diseases](#)” provided by M de la Porta ([IRCCS Clinical Institute Humanitas, Rozzano](#)); “[EVIDENCE project: Erythrocytes properties and viability in dependence of flow and extra-cellular environment](#)” provided by L Kaestner ([Saarland University](#)) and E van den Akker ([Sanguin Blood Supply Foundation](#)); [The International Haemoglobinopathy Research Network \(INHERENT\)](#) provided by P Kountouris ([Cyprus Institute of Neurology and Genetics](#)). The two registry platforms under development in the frame of the network were presented by M Mañú-Pereira ([ERN-EuroBloodNet Scientific Director, Hospital Universitari Vall d'Hebron](#)) : “[European rare blood disorders platform \(ENROL\)](#) and [the Red Blood Cell-COVID19 Platform](#)”. One of the applications of registries is the promotion of clinical trials for the development of new treatments for rare haematological disorders. According to this, [ERN-EuroBloodNet](#) is also working for the organization of Clinical trials as presented by P Fenaux ([ERN-EuroBloodNet Scientific Director, Assistance Publique – Hôpitaux de Paris, Hôpital Saint-Louis](#)).

Main Points highlighted during the discussion by talks:

- [Genomed4all project: Genomics and Personalized Medicine for all through Artificial Intelligence in Haematological Diseases](#)
 - ✓ Three use cases: Multiple Myeloma, Myelodysplastic syndrome and Sickle Cell Disease
 - ✓ Additional members from [ERN-EuroBloodNet](#) will have the opportunity to be involved in [Genomed4all](#) as associated members. The application form is under development.
 - ✓ Experts from [ERN-EuroBloodNet](#) willing to participate in [Genomed4all](#) can collaborate through the SCD survey for existing data.
 - ✓ In the future, the project will include additional use cases.
- [ERN-EuroBloodNet Registries: European rare blood disorders platform \(ENROL\) & the Red Blood Cell-COVID19 Platform](#)
 - ✓ [The COVID19 platform](#) will be permanently open to the experts willing to participate.
 - ✓ The first analysis of the data will be in June.

The closing session was provided by M Mañú-Pereira ([ERN-EuroBloodNet Scientific Director, Hospital Universitari Vall d'Hebron](#)). During the presentation, she informed the audience of different ways to be involved in [ERN-EuroBloodNet](#), and provided the take-home messages.

The [ERN-EuroBloodNet](#) Progress meeting not only gave an update to the community about the development and results of the on-going projects but also enriched interactions among key stakeholders for new key actions in the field of rare haematological disorders. Presentations of the lectures provided are available at the [ERN Collaborative platform](#).

If you are not an [ERN-EuroBloodNet](#) member and are interested in getting the slides please contact maria.rodriquez.sanchez@vhir.org.

THANKS to all for your fruitful participation and valuable contributions!