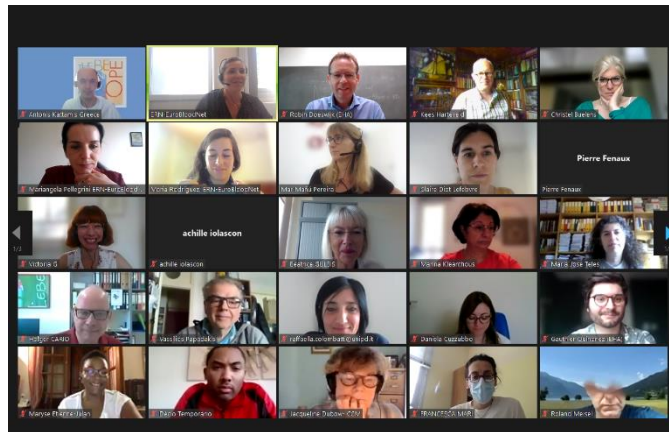


ERN-EuroBloodNet 3rd Progress Meeting Outcomes



19th May 2022, Online



Coordination team:

Pierre Fenaux – Coordinator & Chair of oncological hub

Béatrice Gulbis – Co-coordinator & Chair of non-oncological hub

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Victoria Gutiérrez Valle – Scientific Writer and Digital Activities manager

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Léa Margot – ERN Project Manager

Christel Buelens – Telemedicine & Education Manager

Introduction

On the last 19th of May 2022, more than 100 participants attended the 3rd edition of the [ERN-EuroBloodNet](#) Progress Meeting. This online event has been the occasion to update the [ERN-EuroBloodNet members](#) and [supporting partners](#) on the ongoing actions and collaboration opportunities proposed within the ERN. As a new feature for this meeting, a collaborative table was shared among participants to gather their needs and ideas for developing new concrete actions.

Opening session: ERN-EuroBloodNet status and consolidation

The meeting was initiated with a “*Welcome Message*” provided by the [ERN-EuroBloodNet co-coordinators P Fenaux](#) ([Assistance Publique – Hôpitaux de Paris, Hôpital Saint-Louis](#)) and [B Gulbis](#) ([CUB-Hôpital Erasme](#)). They informed the audience that the ERN-EuroBloodNet has recently been renewed for a period of 1,5 years (until august 2023). With the recent [integration of 36 new members](#), both the disease and country coverages have increased. A new call for extending the disease coverage will be open by the European Commission (EC) in January 2023.

J Valverde, Policy Officer at [DG-SANTE, European Commission \(EC\)](#), provided the second talk on “*ERN’s developments and consolidation*”. He presented the new [European Partnership on Rare Disease](#) developed by the European Commission to increase impact of rare diseases research results. As stated in the [ERNs implementing decision 2014/287/EU](#), an evaluation is going to be launched in September 2023 to assess the performance of ERNs and their members. Finally, J Valverde described the future financial scheme for ERN that is expected to be increased, multiannual and simplified.

To close this first session, [V Gutiérrez Valle](#) ([Scientific Writer & Digital Activities Manager, Hospital Universitari Vall d'Hebron](#)) presented the results of the “*ERN-EuroBloodNet’s 5 years implementation*” with thorough overview of the main activities of the ERN developed so far.

Highlights



ERN Renewal until
august 2023



36 New members
integrated



Upcoming evaluation
in sept. 2022



Increased budget



New European rare
disease partnership
under development

Discussion Highlights:

- There are inequalities among European countries in **prevention and carrier detection in rare diseases** such as Thalassemia or SCD: actions should be taken on this area.
- **Patients associations** can be involved in the ERN-EuroBloodNet through different ways; They are invited to contact the coordination team and/or EURORDIS to join the ERN-EuroBloodNet community.
- Concerns were raised to the EC regarding the **implementation of the In-Vitro Diagnosis Regulation**: the new administrative burden represents a big challenge in the diagnostic process of rare diseases.

Session I: Cross border health & Telemedicine

[R Colombatti \(AO Padua\)](#) opened the first session of the meeting with a presentation on the “**Mapping of expertise and experts & Highly Specialized procedures**”. She presented the different action undertaken in the Cross Border Health Field of Action (TFA), such as the 4 Mappings of highly Specialize procedures or the Pilot on Cross-border collaboration agreement for BMT. Experts have also been invited to update their Expert profile on the [ERN-EuroBloodNet website](#) and to provide any suggestion for a new mapping of expertise need.

The next talk was provided by [P Bartolucci \(Assistance Publique-Hôpitaux de Paris, Hôpital Henri-Mondor\)](#) on “**Improving EU Access to Gene Therapies**”. He explained how this new treatment represents a new area in the European medicine with challenges such as the industries’ dependency, scarce ongoing trials, problems of costs, etc. However, the EU has good research, evaluation, and healthcare systems, and important patients cohorts. Gene therapies represent a new hope in curing severe genetic diseases and require a fast evolving strategy in Europe.

[B Gulbis \(CUB-Hôpital Erasme\)](#) presented some updates regarding the “**New CPMS financial scheme**”. The expertise provided by HCPs on CPMS panels can now be reimbursed (200€/panel). Interested experts to launch a Virtual board to benefit this new financial scheme can contact the coordination team.

M Piggin ([ERN-EuroBloodNet ePAG](#), [PNH Support](#), [PNH Global Alliance](#)), closed the session with a talk on “**Best practices for assisting patients in accessing expensive treatments**”. She explained the procedure developed within the [ERN-EuroBloodNet](#) to assist patients dealing with Cross-border Health Procedures. Members are encouraged to utilise this collaboration model between patients organisations and [ERN-EuroBloodNet](#) for providing information to patients, searching for expert centres or association, and support access to treatment.

Highlights



4 Mappings Highly specialized procedures conducted



Need of European action for gene therapies



New financial support for the CPMS



ERN's model for patients' access to specialized treatments

Discussion Highlights:

- **Gene therapies:** Proposal of creation of a multi-stakeholders workshop to study the feasibility of the pre-clinical and clinical trials in gene therapies. This action could help to raise awareness and maybe fundings on this issue. Teams and experts that want to share a common project at the European level are welcome.
- **Expensive drugs:** The ERN-EuroBloodNet could help on the access to very expensive drugs (such as Eculizumab, Crizanlizumab, Luspatercept and others expensive drugs) and to share knowledge on their effectiveness. The launch of multi-stakeholders workshops on the topic and of a mapping exercise on available drugs could be very useful. EPAGs involvement is crucial.
- **Pharmacoeconomics** could represent a challenge for which the ERN-EuroBloodNet could provide support.
- **Usage of the CPMS** should be encouraged among experts while its use should be simplified by the EC, especially for the new version currently on design.

Session II: Education and Best practices

Highlights

The second session of the meeting was opened by [M. Pellegrini](#) ([ERN-EuroBloodNet Educational & Patients Program Manager, Assistance Publique – Hôpitaux de Paris, Hôpital Saint-Louis](#)) presenting “Short stays & Educational actions”. She informed the [ERN-EuroBloodNet members](#) about the current open call for [preceptorships](#) on highly specialized procedures in Rare Haematological Diseases (RHD). Members are invited to use this opportunity if they want to welcome or participate to one of these clinical based and accredited trainings. To address educational gaps in the field, the [ERN-EuroBloodNet](#) also develops many [webinars](#) programs, both for health professionals and for patients/patients associations. An e-learning platform on Rare Anemia will also soon be accessible through the [ERN-EuroBloodNet website](#).



Open call for preceptorships

The second talk entitled “Guidelines and other Clinical Decision Support Tools” was provided by [L. Malcovati](#) (ERN-EuroBloodNet chair, [Foundation IRCCS Policlinic San Matteo, Pavia](#)) & N Bolaños ([Lymphoma Coalition](#)). It has been explained that one of the first aimed achieved in the field of Best Practices has been the creation of a repository of Guidelines, available on the [ERN-EuroBloodNet website](#). The development of Clinical Practice Guidelines (CPGs) can be done either through the EU-supported program or through the only ERN’s initiative. Any suggestion on prioritization of topics for development of CPGs and CDSTs are welcome. A dissemination plan for CPGs and CDSTs is also planned for the future. Finally N Bolaños stressed out the importance and the benefits of patients’ involvement in the development of Guidelines and Other Decision Support Tools. This inclusion ensures the usability and a relevant impact for advancing patient-centered and trustworthy guidelines.



Development of CPGs opportunities

Discussion Highlights:

- **Education:**
 - Webinars programs for patients and health professionals represent an important achievement that should be further developed.
 - Educational gaps have been identified for the development of new webinars and/or patients-centered information such as: AI intelligence opportunities for patients; patients journey in haemophilia and SCD.
- **Best practices:**
 - New born screening in haemoglobinopathies needs to be promoted
 - CPGs and CDSTs should be identified and/or developed to support to diagnosis of inherited platelets disorders & Von Willebrand disease

Session III: Clinical trials & Research

The Clinical Trials & Research session was opened by a presentation on the “**GenoMed4all Project**”. The aim of this project is to provide evidence on the use of AI for the improvement of patients management. The talk was provided by F Alvarez, M Della Porta ([IRCCS Clinical Institute Humanitas - Rozzano](#)), and M Mañu Peireira ([ERN-EuroBloodNet Scientific Director](#), [Hospital Universitari Vall d'Hebron](#)). They respectively presented the European Federated Platform for AI currently developed in the context of the project, and two ongoing use cases. Since IA technologies are flexible and adaptable, they can be applied to different clinical context and be of high value for the ERN community. A strategy is being developed for the involvement of other ERN-EuroBloodNet members within the [GenoMed4all project](#).

Updates on the “**ERN-EuroBloodNet registries**” were then provided by M Mañu Pereira ([ERN-EuroBloodNet Scientific Director](#), [Hospital Universitari Vall d'Hebron](#)). She reminded the [European commission strategy](#) for promoting the interoperability of registries, with the aim to cope with the fragmentation of the data. Different registries and pilots are being developed under the umbrella of the [ENROL registry](#), such as the [RADeep platform](#), the [Collaborative Platform on RBC and Covid-19 patients](#), and two pilots on BPDCN and Von Willebrand disease. Experts were invited to collaborate in this initiative not only for creating new registries, but also to increase interoperability at the European level.

A Rath provided a talk on the “**ERICA Project**”, which constitutes a support action made for the 24 ERNs to strengthen their research activities and innovation capacities. In particular, the WP3 aims to identifying clinical outcome assessment tools that are patients centers (PCOMS/PROMs) and to develop a central repository. So far two workshops were performed to present the work but also to receive feedback from participants (including EPAGS). Interested members can be involved in the WP3 expert group that is being constituted.

Finally, [P Fenaux](#) ([Assistance Publique – Hôpitaux de Paris, Hôpital Saint-Louis](#)) made a presentation on “**The use of drugs for rare haematological diseases in the context of clinical trials**”. He reminded that the ERN-EuroBloodNet remains available to endorse and assist members in the development of clinical trials.

Highlights



Development of a european federated platform for AI



New pilots registries in Von Willebrand and BPDCN



Development of a PCOMS/PROS repository



Clinical trials opportunities

Discussion Highlights:

- **Development of PROMS** represents a high value. However, these instruments are not all free of access and costs might constitute a break in the use of these kind of tools.
- **Clinical trials:** Patients' involvement in clinical trials development is essential.
- Participants expressed their interest in some specific **research areas** such as:
 - Artificial intelligence
 - MDS response of patients to COVID Vaccines and medication
 - NGS sequencing for inherited thrombocytopenia
 - Promotion of phase 4 studies with economic and social issues
 - Patients-Centered Research

Closing Session

The last part of the meeting was devoted to the presentation of the dissemination tools developed by the [ERN-EuroBloodNet](#) and the different communication channels available to “**Increase the visibility of members’ project**”. The talk was provided by M. Rodriguez Sanchez ([Dissemination Manager](#), [Hospital Universitari Vall d'Hebron](#)) who stressed out that the [newsletter](#) is the first instrument to stay updated on the main opportunities and projects of the ERN. Updates are being implemented on the [ERN-EuroBloodNet website](#) to increase the visibility of ERN-EuroBloodNet actions and experts. Members were invited to update their profile on the [website](#).

Highlights



Experts profile to be update on ERN-EuroBloodNet website

Conclusion:

Many inputs and fruitful discussion resulted from this 3rd ERN-EuroBloodNet Progress Meeting. It has also been the occasion to see all the progress made in 5 years of implementation and the next opportunities for cooperation. The ERN-EuroBloodNet welcomes any suggestions for future areas of joint working and the network is very much looking forward to continuing the excellent collaborations.

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.

Next Progress Meeting will be held the 7th of November (Online)
=> SAVE THE DATE!