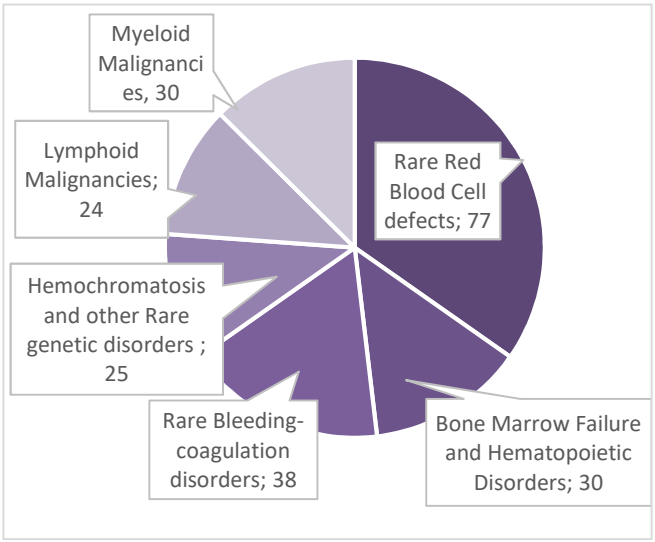
ERN-EuroBloodNet 5th Progress Meeting

9th of November 2023

Outcomes

127 Participants from 17 European Countries !



On the 9th of November 2023, the ERN-EuroBloodNet held its bi-annual Progress Meeting online, gathering 127 participants from 17 European countries. It was a great pleasure to welcome our ERN members, ePAGs, EURORDIS, and other invited stakeholders to this event, as the European Hematology Association (EHA).

The event kicked off with a welcome message provided by the ERN co-coordinators who presented the results from the recent 5 years Evaluation process, as well as the upcoming 4 years framework under the new ERN grant and relevant stakeholders. In addition, the new incorporations to the ERN-EuroBloodNet coordination team were presented to the audience.

Holm Graessner, Coordinator of the ERN on Neurological Diseases (ERN-RND), and currently coordinator of the ERNs Coordinators group, presented the **strategic vision of the ERNs and the direction to be pursued in the upcoming 4-year period**. He highlighted the challenges ERNs face, such as harmonizing their ongoing actions. The focus remains on consolidating the ERN brand through excellent core activities and the delivery of highly specialized procedures.

Closing the Opening session, Yanis Mimouni, Senior Project Manager of the European Joint Programme on Rare Diseases (EJP-RD), presented the **upcoming Rare Disease partnership: ERDERA.** This partnership will consolidate the rare disease research ecosystem as a continuation of current EJP-RD. It will ensure a link with the upcoming **Joint Action on integration of ERNs into national healthcare systems (JARDIN)** and pave the way for ERNs integration in the national healthcare systems and increase their sustainability.

The Progress Meeting then tackled different topics split in two main sessions:

ERN-EuroBloodNet strategic vision: Looking back and forward

The Coordination Team and the leaders of the actions showcased during the session the main successes across different fields of actions:

Best Practices:

- The repository of Clinical practice guidelines (CPGs) and other decision support tools (CDSTs), as well as the status of development of the CPGs and CDSTS produced by the network on Burkitt Lymphoma, Vexas Syndrome and Sickle Cell Disease were detailed to the audience.
- The added value and results from the EU Mappings for the availability of highly specialized procedures conducted, including the latest one on Management of Venous Thrombosis and Arterial Thrombosis in Pediatric patients were also presented.
- Lastly, the creation of patients' pathways from patient therapeutic educational program was showed including the current pilot for von Willebrand Disease.

New actions proposed:

- ✓ Update of the CPGs for laboratory diagnosis on Hemoglobinopathies
  - Diagnosis is also covered in the Recommendations on Sickle Cell Disease being produced by the network.
  - Collaboration can be also established with Haemoglobinopathies in European Liaison of Medicine and Science (HELIOS) WP1 on Molecular research and diagnosis in hemoglobinopathies.
- ✓ New CPG in bleeding disorders, considering recent development of new therapeutic options.
- ✓ Patients' journeys on myelodysplastic syndromes.
- ✓ Monitoring of the implementation of CPGs and CDSTs at national level:
  - New Key Performance Indicators (KPIs) will be identified and mapped through EU mapping exercises during the next 4 years frame.
  - Inclusion of KPIs in ENROL registry dataset will be also explored.

Education:

- Main results from the educational actions ongoing were presented into two main targets for health professionals, with the webinars programs, preceptorships and eLearning platform, and for patients, with the ongoing trainings and new program on transition.
- The ongoing collaborative programs for education on research were also introduced to the audience, with the concrete collaborations with ENROL Registry for the development of webinars program & knowledge pills for patients, with GenoMed4ALL for the Educational program on Artificial Intelligence in hematology for public-at-large, and the educational programs to be launched under the collaboration with the recently granted projects Assessing efficacy and safety of genome EDITing approaches for Sickle Cell Disease (EDITSCD) and Haemoglobinopathies in European Liaison of Medicine and Science (HELIOS).

New actions proposed:

- ✓ New webinar program for pediatric thrombotic disorders.

Cross Border Health & CPMS:

- ERN-EuroBloodNet activity on the Clinical Patient Management System (CPMS) as well as the status of the development of the new version of the platform were showed to the audience.
- The two new engines implemented at the website to increase the searchability of ERN-EuroBloodNet healthcare providers and health professionals expertise and activity were showed: the Disease Cards, pooling reference centres and experts, materials (as CPG and CDST, webinars, etc.) and actions by 77 RHD disease groups; and the PDF generator integrated in the repository of RHD experts and facilities to allow the download of reports by the search criteria.
- ERN-EuroBlooNet Patients Assistance InfoPoint has been implemented at the website as an online form for the structured compilation of queries from RHD patients requiring assistance for cross border health rights.

New actions proposed:

- ✓ Virtual meetings to promote the completeness of ERN-EuroBloodNet Disease cards and expert profiles. Both tools serve to consolidate and make the expertise and activities of the network easily accessible.
- ✓ Convincing hospital Chief Executive Officers and national authorities is crucial to guarantee the translation of ERNs' actions into national health systems.
    - JARDIN will consolidate and expand mechanisms and strategies to ensure integration of ERNs actions into national health systems level.
    - A webinar was proposed to explore strategies for bridging the gaps between ERNs and health systems.

ERN-EuroBloodNet advancing on clinical research

Including the presentation and discussion on topics such as the collaboration with the European Medicine Agency (EMA) on patient's registries, the new EU Directive on Pharmaceuticals, and the improvement of access to diagnosis and treatments.

Regulatory aspects of rare disease patients' registries: Pathway to cooperation with EMA

- Carla Jonker from the EMA outlined the process for obtaining EMA qualification of ERNs registries, emphasizing its significance for regulatory and decision-making purposes.
- Three collaborative actions with ENROL registry illustrated how ERNs registries can improve the quality of data for EMA validation and re-use, including: the validation of synthetic data through GenoMed4All and SYNTHEMA, the re-use of data in clinical trials through IMPACT-AML and the use of PROMs in reimbursement decision making process through RD Catalyst grant.
- Simone Boseli, EURORDIS public affairs representative, provided an overview of the key changes outlined in the proposal for the new Directive on medicinal products for human use. The presentation highlighted the potential impact of approving the proposal on the development of new drugs.

ERN-EuroBloodNet improving access to diagnosis and treatments

- ERN-EuroBloodNet is currently sponsoring clinical trials, with three proposals in progress. The first contract for a EuroBloodNet sponsored clinical trial has been signed, focusing on the safety and efficacy of Mitapivat in adult patients with erythrocyte membranopathies (SATISFY). The trial was presented to the audience.
- Conducting EU mappings to assess the accessibility and availability of treatments for very rare diseases is crucial to understand the actual availability of such drugs at the national level. Use cases from the mapping exercises focusing on access to drugs for Paroxysmal Nocturnal Hemoglobinuria and Myeloproliferative Neoplasms disorders were presented.

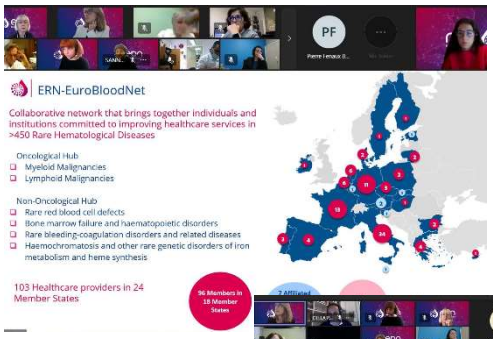
Key conclusions:

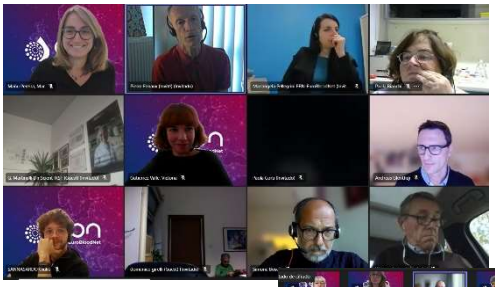
- ✓ EMA's qualification of ENROL Registry will enhance its capacity to gather high-quality data, serving as the basis to advance our understanding of rare hematological diseases. This presents a genuine clinical opportunity to elevate the quality of our efforts within the clinical network.
- ✓ Generation of synthetic data starting from retrospective data is a powerful tool. It provides sufficient clinical fidelity in reproducing populations that can be used as controlled arms for randomized studies.
    - Establishing a link with regulatory agencies is crucial. This allows to better understand their views on the most relevant and effective approaches to ensure valid clinical research and trials.
    - Quality of real data is essential to provide high quality synthetic data. Data sources must be associated with comprehensive information on both clinical and genomic features.
    - The broad adoption of this technology is feasible as it relies on generative artificial intelligence, utilizing algorithms that do not require high computational power.
- ✓ Attaining high-quality data from Patient-Reported Outcome Measures (PROMs) is a challenge due to low adherence. Exploring alternative methods becomes essential to enhance adherence and standardize data collection, thereby mitigating issues related to missing data and bias.
- ✓ Clinical trials centralization can sometimes complicate the process rather than simplifying it. Reducing the duration of exclusivity for companies might discourage them from entering the European market.
    - Member States have chosen not to actively participate in drug regulation, placing the responsibility entirely on companies. Unfortunately, these regulations may, at times, lead to a decrease in the availability of drugs rather than an increase.
    - ERNs can be a powerful tool to provide evidence-based suggestions to policy makers.
    - Ensuring patient involvement is crucial, and EURORDIS has an opportunity to request the European Commission to provide clearer explanations regarding engagement practices.

Thank you all for your active participation!

Next ERN-EuroBloodNet meeting will be held face to face at European Hematology Association (EHA) Congress!

See you 13th June 2024 13:00-16:00 in Madrid!





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