ERN-EuroBloodNet
7th Progress meeting

6th November 2025, online

ERN | EuroBloodNet

European Reference Network | Hematological Diseases (ERN EuroBloodNet)

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ERN-EuroBloodNet 7th Progress meeting was held 6th November 2025 online. **148 participants from 67 ERN-EuroBloodNet Healthcare providers and 18 EU Member States** registered to the meeting. 77% of participants were members representatives or part of the multidisciplinary teams, while 3% were ePAGs and patients' representatives (Fig 1). The most represented areas of expertise were red blood cell disorders and bone marrow failures (Fig 2).

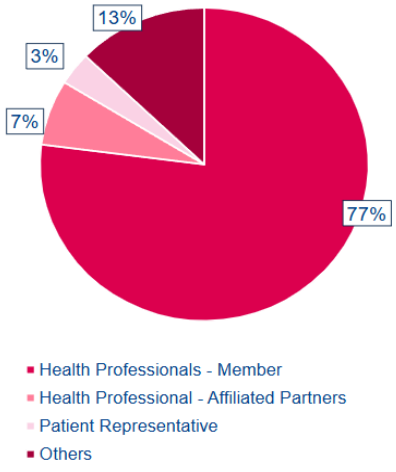

Fig 1. Role of participants of the meeting

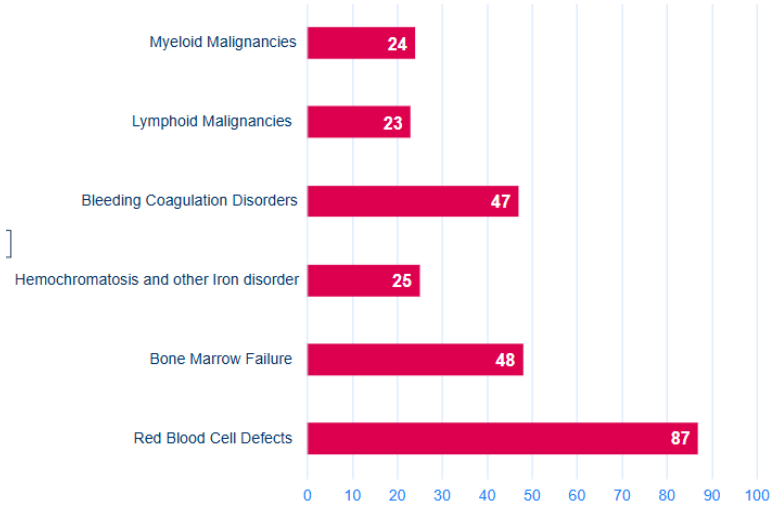

Fig 2. Subnetwork of Expertise of Participants

The event kicked off with a welcome message provided by **Pierre Fenaux** (ERN-EuroBloodNet coordinator, AP-HP – Hôpital Saint Louis, Paris) and **Béatrice Gulbis** (ERN-EuroBloodNet co-coordinator, H.U.B. Hôpital Erasme – ULB, Brussels) who presented the current overview of the ERN relevant stakeholders and agenda for the day.

Opening plenary session

Maria del Mar Mañú Pereira (ERN-EuroBloodNet co-coordinator, Vall d'Hebron University Hospital, Barcelona), actual ERN Coordinators Group (CG) vice-chair, gave an overview of the actual ERNs state of play. She highlighted that no short-term call for new full ERN members is foreseen, and the next opportunity in 2026 will be limited to Affiliated Partners in countries still lacking ERN-EuroBloodNet coverage, specifically Croatia, Latvia, Romania and Norway.

She also stressed the importance of long-term sustainability, noting that the current EU Multiannual Financial Framework (MFF) runs until August 2027, while preparations for the next MFF (2028–2034) are already underway. Coordinators are engaging proactively to ensure ERNs' continued visibility, impact, and financial support.

In terms of strategic outreach, she described joint efforts with DG SANTE to strengthen communication and promote ERN success stories, including achievements in patient registries, patient support, education, and clinical guidelines. Finally, she announced that registration is now open for the 9-11 December 2025 High-Level Meeting on Rare Diseases, featuring EU policymakers, ERN leaders, patient representatives, and major EU projects such as ERDERA and JARDIN.

Stefano Vettorazzi (Policy Officer at the European Commission and co-chair of the ERNs Working Group on Knowledge Generation and Capacity) gave a key note speech on the transversal ERN working groups. He presented the updates on the Knowledge Generation and Capacity group's activities, including the new AI & Rare Diseases subgroup. He highlighted the WG's broad scope, covering Erasmus+ collaborations, the ERN Academy curriculum, and shared education repositories.

With 23 of 24 ERNs participating, the AI subgroup is the most active, involving 14 ERNs, including ERN-EuroBloodNet, which also sits on the Steering Committee. A series of papers are already in development, with first drafts under review and contributions expected from 12 ERNs.

Stefano Vettorazzi emphasized that high-quality, standardised data is key for AI tools to meaningfully support clinical practice in rare diseases.

The plenary session closed with **Cécile Ollivier** (ERDERA WP9 co-chair, Critical Path Institute) keynote speech on ERDERA's work to develop regulatory-grade real-world evidence (RWE).

European Rare Disease Research Alliance (ERDERA) aims to create a Clinical Research Network to:

- Leverage facilities and expertise to address these challenges by creating, integrating, and processing real-world RD cohort data for clinical trials.
- Develop a platform for the development and validation of regulatory-grade patient-reported as well as indirect Clinical outcome assessment for RD
- Develop digital tools allowing efficient and broad collection of patient self-reported data in RD and integration with patient databases

The role of Real-World Evidence (RWE) in enriching benefit-risk evaluations, underscoring its capacity to augment traditional trial findings and inform healthcare decisions was also discussed with the audience.

ERN-EuroBloodNet contributes especially to WP9–WP10 interfaces on registries, longitudinal data, PROs/PREMs and multimodal integration. ERDERA's WP9 defines how datasets can meet regulatory standards, with early case. A new AI/ML-based new born screening module is being built using controlled vocabularies to enable comparison across EU programmes. Data will be harmonised and integrated into a virtual platform, aligned with future EHDS interoperability. ERDERA is in active dialogue with EMA to ensure methods meet regulatory expectations.

ERN-EuroBloodNet website navigation

Daiana N. Lopez (ERN-EuroBloodNet's dissemination manager, Vall d'Hebron University Hospital, Barcelona) presented the ERN-EuroBloodNet website as a central access point for key resources including the:

- EDU Blood Academy

Not only for upcoming webinars, but also to make the most of the materials from past sessions. All presentations and recordings are available, so you can watch them on demand and share the content with colleagues and patients.

- ERN-EuroBloodNet YouTube channel

To find webinars, patients' testimonies and other informative/educational videos developed by the network.

- CPGs/CDST repository

A comprehensive repository of CPGs and CDMTs available online, classified by Quality Domains and to identify gaps for promoting the creation of new ones on areas where they are lacking.

- Experts repository

Closely connected to the development of Clinical Practice Guidelines, she emphasizes the importance of maintaining the "Experts Repository" and keeping the profiles up to date. Whenever new CPGs need to be developed or expert consultations are required, only those whose profiles include the relevant subnetwork of expertise and disease coverage remain listed.

Daiana encouraged attendees to take full advantage of the available tools and information and update their expert's profile as the Expert Repository is our main tool to identify and extract expert lists by subnetwork and disease area.

Clinical Practice Guidelines & Access to Highly Specialized Care

ERN-EuroBloodNet is developing Clinical Practice Guidelines (CPGs) and other Clinical Decision Support Tools (CDSTs) in the field of rare hematological diseases (RHD) in collaboration with the EC and the main hematologic networks and associations.

During this session **Béatrice Gulbis** (ERN-EuroBloodNEt co-coordinator, Hôpital Erasme – ULB, Brussels) presented the ERN-EuroBloodNet's new CPGs and CDSTs and highlighted a systematic comparative analysis of national SCD guidelines in EU and UK to identify any differences and commonalities between practices, but also to identify any evidence gaps. She gave two examples of the work in progress. One of them emphasized the transition from adolescence to adult care for which limited recommendations are proposed and force to the conclusion that improvement is needed. This revision on SCD recommendations is being designed not only to improve disease-specific management but also to address systemic care gaps, with a strong focus on real-world applicability, structured care pathways, and coordinated ERN dissemination and education efforts.

The presentation was closely linked to the next presentation given by **Mariangela Pellegrini** (ERN-EuroBloodNet Health Policy, Education and Patient Engagement manager, AP-HP – Hôpital Saint Louis, Paris) who introduced the newly launched Charter for optimal care transitions from pediatric to adult care in Sickle Cell Disease, recently showcased at the European Parliament, which was created to address systemic barriers in the shift from paediatric to adult care, including fragmented handovers, lack of protocols, lost medical records, insufficient consultation between teams, and social or discriminatory challenges. With more patients surviving into adulthood, transition failures (not medical limitations) now drive poorer outcomes. The Charter provides a structured framework to standardize transitions, strengthen continuity of care, and served as a proposal for implementation in other national regulation centres and national systems of care.

Education & Collaborative programs

Mariangela Pellegrini (ERN-EuroBloodNet Health Policy, Education and Patient Engagement manager, AP-HP – Hôpital Saint Louis, Paris) gave an overview of the current ERN-EuroBloodNet actions for health professionals and patients:

- Health professionals including three strands: on-site preceptorships, single-session webinars on cutting-edge topics, and multi-webinar "Topic on Focus" programmes; most professional courses are EBAH-accredited and increasingly run in collaboration with European scientific societies (e.g., EHA).
- Patients developing tools and programs that empower them through education, enabling better disease understanding and management. Offer with on-site training, single-topic webinars, and comprehensive programmes developed with research projects to train patients not only in therapeutic education but also in research.

In addition, there was a team update with the introduction of **Hiba Fadlallah** as Education & Patient Program Manager and **Christophe Goetghebeuer** as Health Professional & CPMS Project Manager.

As an example on a collavorative program for education on research, **Annarita Miccio** (Institut Imagine, Paris) presented an update on EDIT-SCD, a multi-session educational webinar program on gene-editing approaches for Sickle Cell Disease. The series supports therapeutic awareness and preparedness in countries with varying readiness for clinical trials. It compares different gene-therapy and gene-editing strategies with a focus on efficacy and safety, and features an academic approach advancing through preclinical stages. Annarita Miccio highlighted the link between education and broader advocacy for equitable therapeutic access across ERNs. The program is designed with patient-friendly communication in mind, using lay-language guidelines, patient input, and medical-writer support to ensure clarity and visual accessibility. Ongoing feedback is integrated into future sessions, and dissemination occurs through ERN channels, newsletters, the website, and institutional and speaker social media.

CPMS

Clinical Patient Management System (CPMS) 2.0 has been designed to improve both functionality and user experience in assisting ERNs to enhance the diagnosis and treatment of rare or low-prevalence complex diseases across Member States.

Christophe Goetghebuer (Health Professional & CPMS Project Manager, Hôpital Erasme – ULB, Brussels) presented updates on CPMS 2.0, highlighting a new platform that allows ERN and non-ERN clinicians across the EU and Norway to submit cases and provide expert advice, broadening access to specialist input. The main messages from the coordination team were to have an overview of the CPMS 2.0 registration, use and satisfaction through a survey (access the survey here), to help users for registration within the CPMS 2.0 platform as well as to submit difficult cases by giving access to experts through virtual boards (scheduled meeting of experts in a specific field). Projects to integrate CPMS into national electronic health records are underway under JARDIN supervision, and an open-source code will be delivered in 2026, spreading the CPMS model beyond ERN networks. The underlying objective is to embed CPMS practice within clinical workflows, from local to pan-European level, enabling cross-ERN consultations for ultra-rare diseases.

Registries & clinical outcome research

Sara Reidel (Rare anemia disorders research laboratory data driven lead, Vall d'Hebron, Barcelona) presented EpiBlood, the European Rare Blood Disorders Platform (ENROL) Registry Module for the Collection of annual counts of RHDs patients. EpiBlood aims to systematically collect standardized information on the annual counts of patients with RHDs through a secured REDCap system. The module supports epidemiological surveillance and planning, enabling analyses by disease, country/centre, and age group.

- Contribution from each HCP is mandatory for the subnetworks where the institution is recognized as ERN Member. The opportunity to contribute is also extended to other subnetworks.
- Next data collection exercise for year 2026 will start on 1st March 2026 until 31st May 2026.
- ERN-EuroBloodNet Members and Affiliated Partners will be contacted during December with the Handbook and PDF template with the information that will be requested for this year campaign.

Clinical trials

Pierre Fenaux (ERN-EuroBloodNet coordinator, AP-HP – Saint Louis, Paris) outlined ERN-EuroBloodNet's emerging role as a sponsor of clinical trials to better serve rare hematological diseases. He noted that industry trials tend to focus on more common indications and maintain tight control over study design, while academic trials often face slow, complex approval pathways and additional obstacles when conducted internationally. An ERN-led sponsorship model can therefore fill critical gaps by prioritising rare conditions and facilitating coordinated, cross-country execution.

ERN-EuroBloodNet's strategy is to assume the sponsor role where unmet needs and innovative therapies align, helping to advance clinical research that might not otherwise be initiated.

Thomas Doeven (University Medical Center Utrecht, Utrecht, Netherlands) presented the SATISFY study, an investigator-initiated, multicentre Phase 2 trial in Denmark and the Netherlands (with a parallel study in Canada), sponsored by ERN-EuroBloodNet and funded by Agios. The trial evaluates mitapivat for hereditary spherocytosis, hereditary stomatocytosis, and CDA type II, addressing a setting with few therapeutic options beyond splenectomy. EuroBloodNet, as sponsor, provided regulatory and operational support: protocol amendments, authority interactions, and safety reporting, while keeping scientific control with investigators.

Thank you all for your participation!

Presentations are available on the ERNs Collaborative Platform and on the ERN-EuroBloodNet website.