ERN | EuroBloodNet

Present and future of the ERNs: HLM4Rare & the European Cancer Mission

María del Mar Mañú Pereira *(Vall d'Hebron Institut de Recerca (VHIR), Barcelona, Spain)*
Matteo Della Porta *(IRCCS Clinical Institute Humanitas di Rozzano, Italy)*
Angelo Loris Brunetta *(Thalassaemia International Federation (TIF), Genoa, Italy)*

European Reference Network | Hematological Diseases (ERN EuroBloodNet)

Funded by the European Union

European Reference Networks (ERNs)

- **7,000 Rare Diseases (RDs),** affecting less than **1 in 2000 individuals**. Due to the small patient number and the often limited knowledge, rare diseases are the area in public health in which joint efforts among Member States (MS) is most justified and crucial.
- RDs are too often underdiagnosed or diagnosed too late, taking an **average of 7 years to get answers**. Less than **10% of RDs have a treatment**.
- **European Reference Networks (ERNs) involve healthcare providers across Europe**. They aim to facilitate discussion on complex or rare diseases and conditions that require highly specialized treatment, concentrated knowledge and resources.
- The first **24 ERNs** covering 24 different medical specialties were officially approved by the EC in December 2016 and started their activity in March 2017, one of them being **ERN-EuroBloodNet**.
- As of October 2025, the ERNs include **1,606 specialized centers** located in **375 hospitals** across 27 Member States and Norway.

European Commission | Health

ERNs roadmap

Governance 24 ERNs □ Coordinators TRIO

24 ERNs COO TRIO	July 2025 - June 2026	July 2026 - June 2027	July 2027 - June 2028
CHAIR	Ruth Ladenstein - Paedcan	**Mar Mañú Pereira - ERN-EuroBloodNet**	Alberto Pereira - ENDO- ERN
VICE CHAIR	**Mar Mañú Pereira - ERN-EuroBloodNet**	Alberto Pereira - ENDO- ERN	tbd
FORMER CHAIR	Lucas SanGiorgi - ERN - BOND	Ruth Ladenstein - Paedcan	**Mar Mañú Pereira - ERN-EuroBloodNet**

MFF (2028-2032)

European Reference Networks

JARDIN

Pilot Network | **ERNs creation** | **ERN implementation** | **ERNs integration** | **ERNs impact**

2003 enerca European Network for Rare and Congenital Anaemias

2017 ebn ERN | EuroBloodNet

ERNs MISSION

2023 European Reference Networks

2027

Professionalization of ERNs

2032

ERNs Actions Delivering

National Healthcare Systems

Statement of the ERN Board of Member States on Integration of the European Reference Networks to the healthcare systems of Member States

ERN's Sustainability beyond 2027

- Multi-frame Grants 2017 = **Current grant 01/09/2023 to 31/08/2027**

EU Budget (MFF) 2028–2034: Overview in a Nutshell

A proposed budget of **€1.98 trillion:** increase from €1.21 trillion in 2021-2027

EU's **multi-annual financial framework (MFF)** in the long-term budget, which sets budget limits and strategic priorities over a seven-year period across multiple funding instruments.

The EU Commissions' Proposal aims to deliver a long-term budget which:

- Is a larger and more flexible budget
- Streamlined EU financial programmes
- Focus on EU collaboration in health and innovation

Impressive € - ERNs must not miss out !

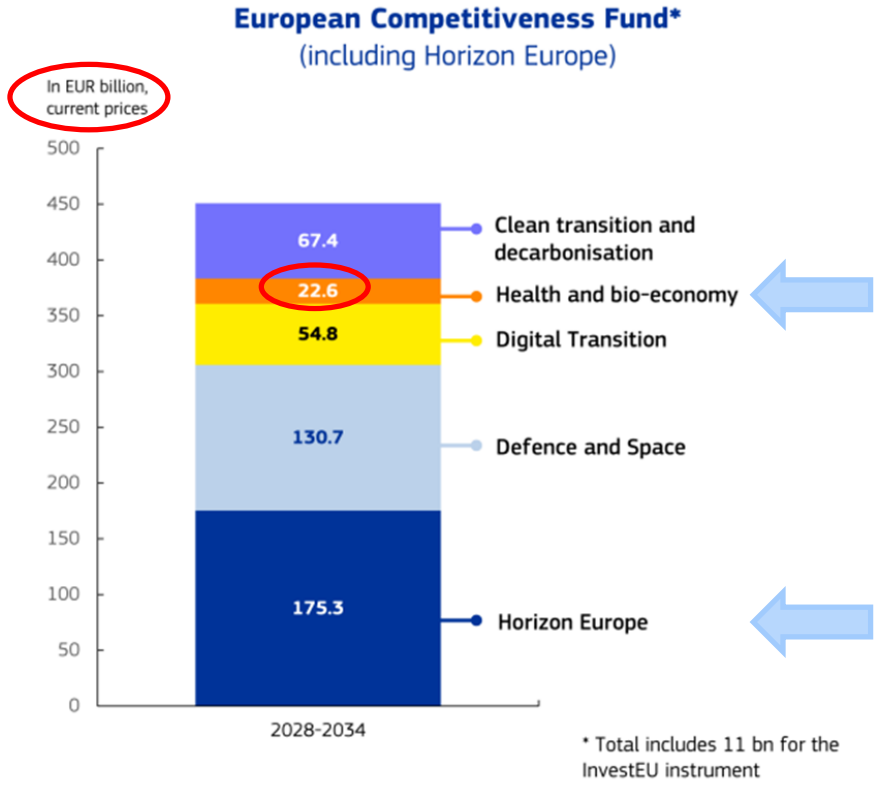
ERN's Sustainability beyond 2027

Health in the New MFF

Health integrated into European Competitiveness Fund (ECF) — replacing EU4Health

ECF Total: **€22.6bn for Health & Bioeconomy**

Health priorities under ECF include **rare disease collaboration, health system efficiency, access to medicines, promoting innovation in biotechnology**



Corresponding threshold for ERNs Budget in MFF 7 year multi-annual grants of at least €110m total, 2028–2034

ERN's Sustainability beyond 2027

EU Budget (MFF) 2028–2034: Overview in a Nutshell

MFF (Multiannual Financial Framework) 2028-2034

(Commission Proposal - 16 July 2025)

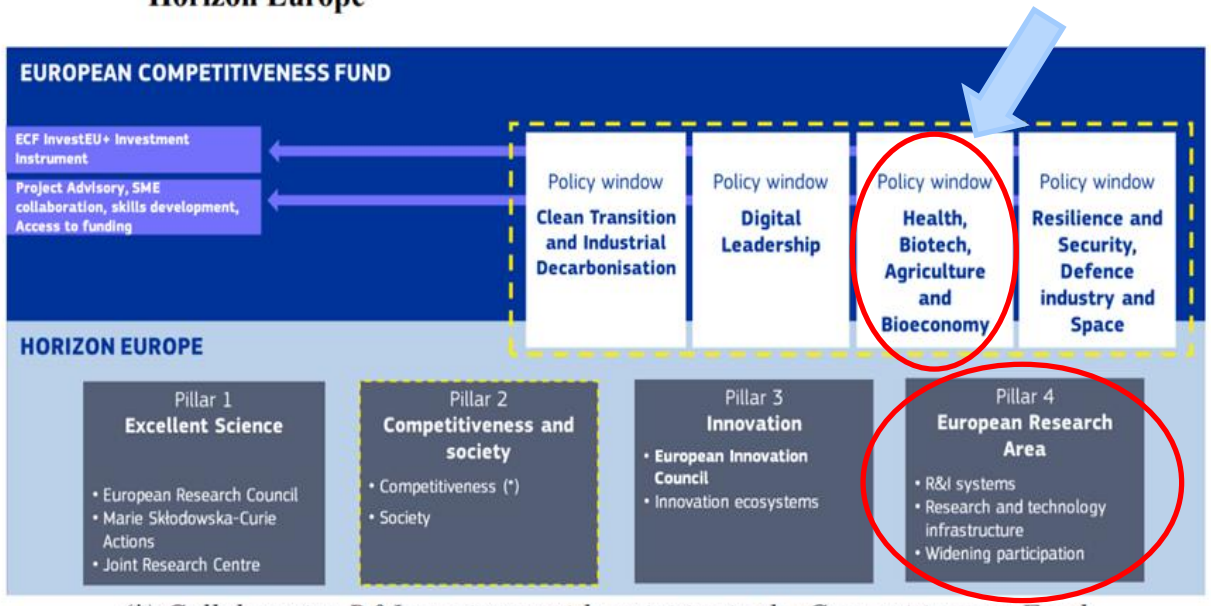
- Phase: beginning of negotiations, release of legislation concerning individual funding programmes.
- 4 MFF Consultations answered in **November.**
- **Council main decision-maker** - Ambassadors needed.

Formal adoptions for the end of 2026/Q1 to Q1/2027.
Contacts to the SANT committee & relevant decision makers ongoing.

ERN's Sustainability beyond 2027

Horizon Europe in the new MFF

II. Boosting European competitiveness: the European Competitiveness Fund and Horizon Europe



(*) Collaborative R&I consistent with activities in the Competitiveness Fund

€175 billion, increase from **€95.5 billion for 2021-2027.**

4 pillars to support research, innovation, attract and nurture research talent, foster international collaboration, and connect science with society

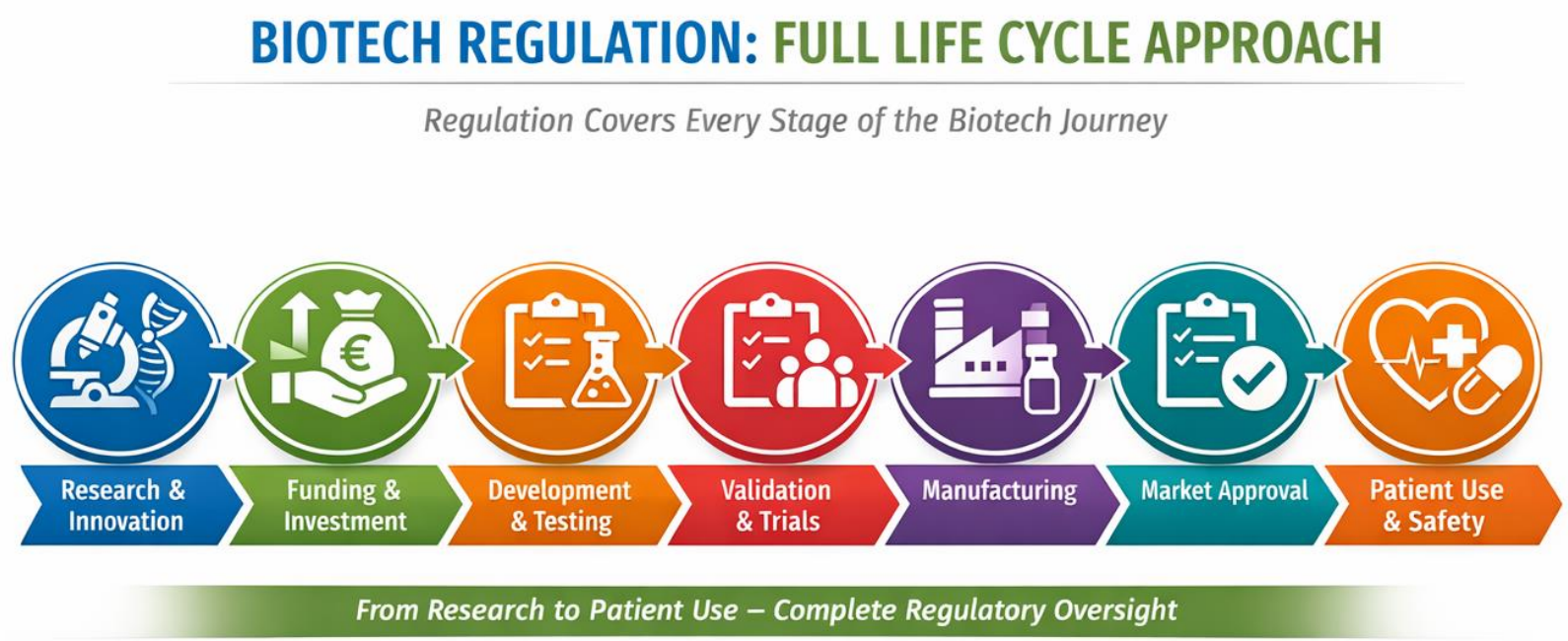
Invest in research and innovation in **regenerative therapy e.g. diverse ATMPs, CAR T cells**, and support **research and innovation expertise**

Will work on health policy priorities of the European Competitiveness Fund.

Biotech ACT

Global objective

Strengthen Europe's biotechnology and biomanufacturing ecosystem across the full life cycle, from research and investment to patient use — accelerating innovation, competitiveness and safe translation into healthcare.

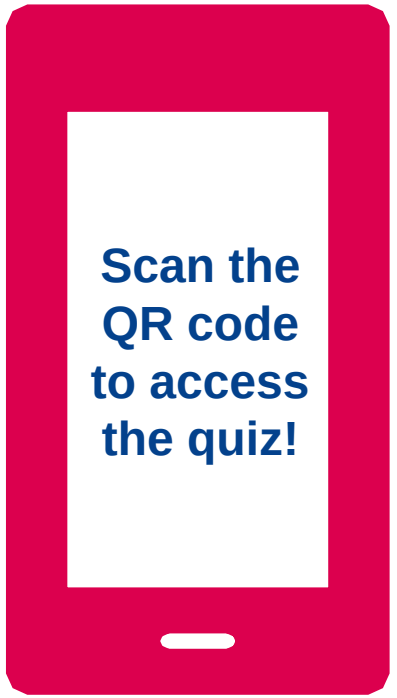


Key pillar based on Regulation & Evidence, Data & Technology, Ecosystem & Competitiveness

- Objectives, Scope and Strategic Direction
- Simplification and Regulatory Acceleration
- Clinical Trials and Evidence Generation
- Innovation-Friendly Regulatory Pathways
- Regulatory Sandboxes and Controlled Testing Environments
- Biotech Data and AI-Ready Infrastructures
- Investment, Scale-up and Industrial Capacity
- Skills, Talent and Ecosystem Collaboration
- Governance, Coordination and Global Competitiveness

Regulatory status: currently a legislative proposal; final provisions may evolve through the ordinary EU legislative process.

Some questions to set the scene



Clinical trials: global map of timelines and regulatory

Executive view 2026. Indicative time to decision / possible start; real start-up may take longer.

Key message: the EU remains slower in multinational trials

Indicative comparison; regulatory clocks do not always reflect the total activation time.

EU
113 days

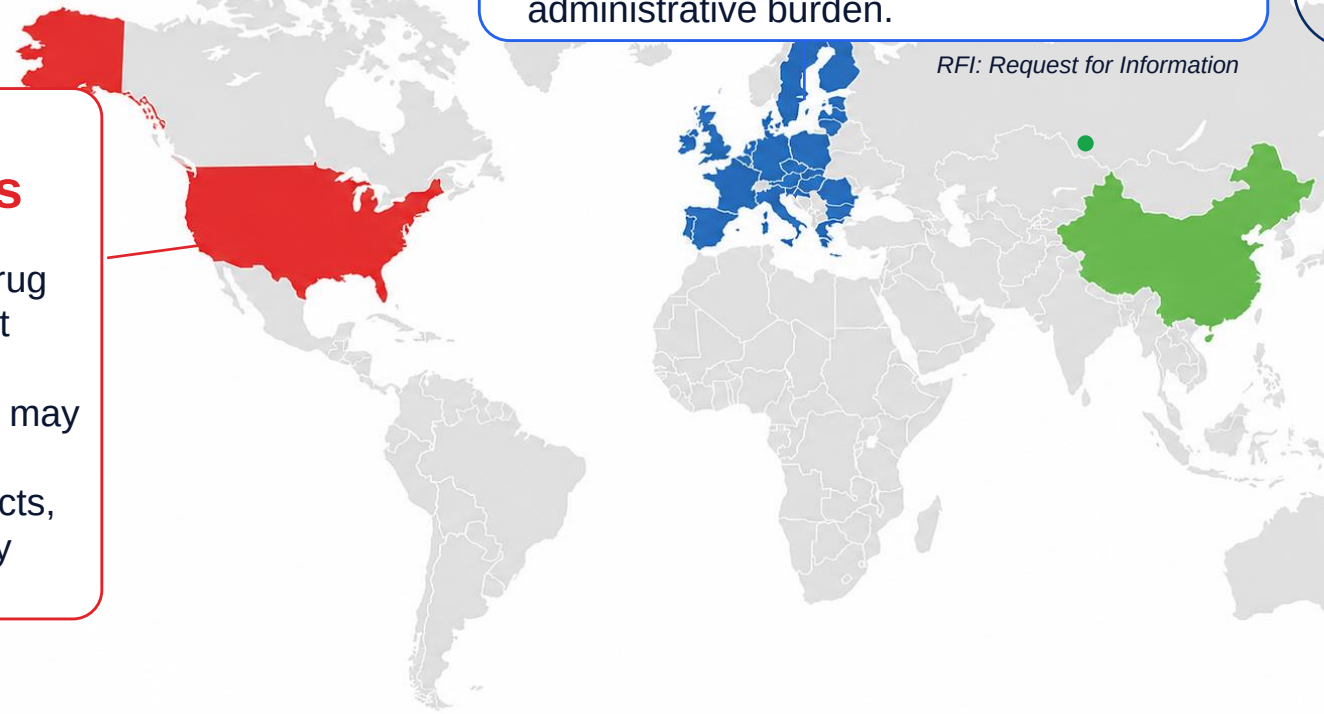
Current average for multinational trials.
Problems: fragmentation across Member States,ethics review + Part II, RFIs, and administrative burden.

RFI: Request for Information

UK
~41 days

Combined MHRA + HRA/REC review benchmark
Problems: NHS contracting, capacity, local set-ip and site activation may delay real start-up

MHRA: Medicines and Healthcare products Regulatory Agency
HRA: Health Research Authority
REC:Research Ethics Committee
NHS: NAtional Health Service



USA
60-47 days

Investigational New Drug (IND): if FDA does not impose
a clinical hold, the trial may start.
Problems: IRB, contracts, and site activation may delay start-up.

IRB: Insitutional Review Board

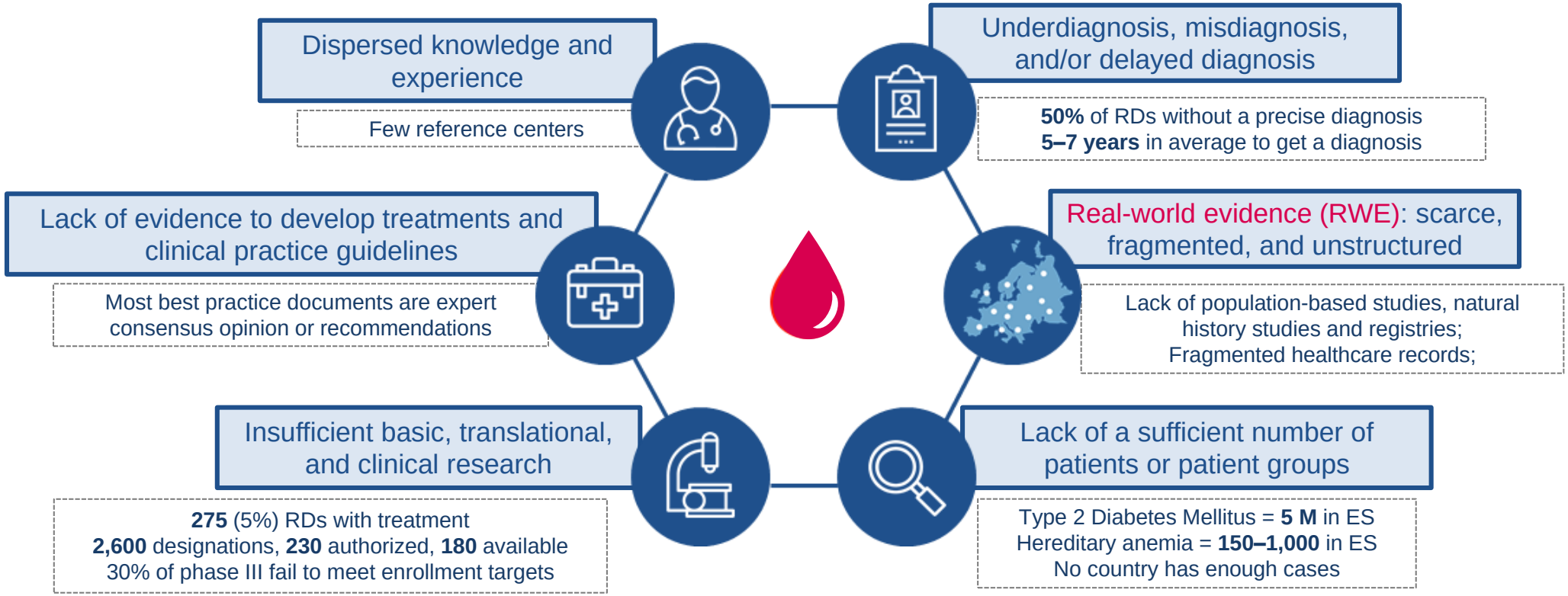
China
~60* days

Implicit approval CTA pathway; 30-working-day route for selected innovative drugs.
Problems: NMPA/CDE requirements, local coordination and region-specific implementation.

NMPA: National Medical Products Administration
CDE: Center of Drug Evaluation

Sources: COM(2025) 1022 — European Biotech Act proposal; FDA IND framework. USA/China values are indicative regulatory benchmarks; operational start-up may take longer.
EFPIA (European Federation of Pharmaceutical Industries and Associations) de 2024; NMPA Announcement No. 86/2025; NIH ClinRegs China.
Launch of clinical trial reforms - GOV.UK

Unmet needs in Rare Diseases (RDs): +7000 RDs, 30Mi EU



fewer than 15% of paediatric rare-disease patients can account for up to 50% of hospital care processes and costs

HLM RARE 2025

The European Reference Networks, MEP Vytenis Andriukaitis (S&D, Lithuania) and the Brains for Brain Foundation, hosted in Brussels from 9-11 December 2025 the first three-day High-Level Meeting on a European Innovation and Care Ecosystem for Rare and Complex Diseases (HLM Rare 2025). A key milestone of this meeting was the launch of a community-led Declaration.

THE DECLARATION ON A EUROPEAN INNOVATION AND CARE ECOSYSTEM FOR RARE AND COMPLEX DISEASES

8 Recommendations

- Prioritise the EU Action Plan on Rare Diseases with a Clear Governance unifying the Rare Disease Community
- Strengthen Workforce Development and Capacity Building for Rare Diseases and formalise the ERN Academy
- Accelerate Equitable Access to Diagnostics and enable Early Treatment Onset with Innovative Orphan Drugs for Unmet Medical Needs
- Foster EU Leadership in Clinical Trials for Rare Diseases through Inclusive Collaboration between Academy, Patient Groups and Industry to accelerate Innovation for people living with rare diseases
- Create, in each Member State, at least one comprehensive rare disease infrastructure cluster (CoRDIC) for research, innovation and care
- Boost real-world evidence generation by making Rare-Disease Data High Quality and Clinical Utility, Interoperable and Integrated under the European Health Data Space
- Explore new business models and mechanisms to prioritise equitable access to innovative orphan therapies and diagnostics, also supported by a European Guarantee Fund
- Ringfence ERNs funding under the 2028-2034 Multiannual Financial Framework

2025 EVIDENCES OF SUCCESS

500 people

250 registered for in-person attendance across the 3 days and + 250 watched the livestreaming each day

+ 80 speakers

including EU Commissioners, MoH, MEPs, and EC and EMA representatives

+2000 organisations & individuals

signed the Declaration during or after the conference

HC Newsletter

Mentions from Politico during the days of our event

36 earned media

mentions on LinkedIn from different key stakeholders, including Emer Cooke and Hans Kluge

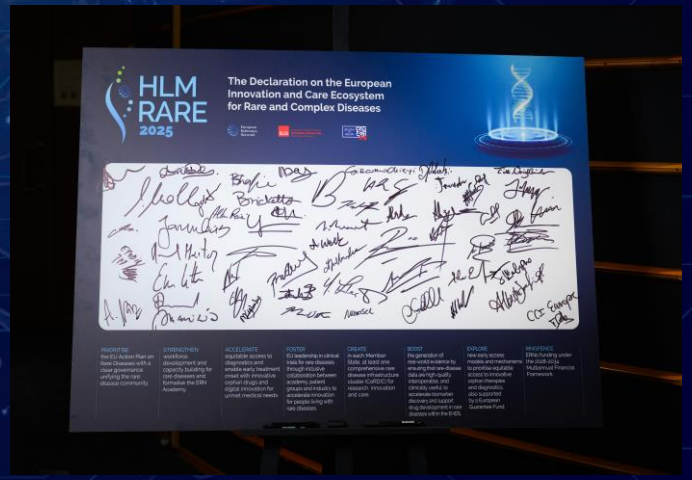
10,214

HLM Rare unique page views in December 2025 alone

Op-ed

Published in the Parliament Magazine following HLM Rare 2025

The EU needs a 28th Regime for life sciences



Signatures collected on the Declaration during the HLM Rare 2025

HLM RARE 2025: SOME OF THE STAKEHOLDERS THAT WERE PART OF IT

EU and national policy- and decision-makers

European Commission

HaDEA

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

MINISTRY OF HEALTH
OF THE REPUBLIC OF LITHUANIA

ESPAÑA
REPRESENTACIÓN
PERMANENTE EN LA
UE

Medical and research community

European Reference Networks

EURORDIS
European Rare Diseases Research Alliance

ISNS
International Society for Neonatal Screening

ILAE-EUROPE

THE CYPRUS INSTITUTE OF NEUROLOGY & GENETICS

Realise D

Inserm

orphanet

EUROPEAN PAEDIATRIC NEUROLOGY SOCIETY

screen4rare

Bambino Gesù
OSPEDALE PEDIATRICO · IRCCS

JARDIN

SJD Sant Joan de Déu
Barcelona · Hospital

Patient community

EURORDIS
RARE DISEASES EUROPE

Childhood Cancer International
EUROPE

IPOPI
INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

CHILDREN'S TUMOR FOUNDATION
ENDING NF THROUGH RESEARCH
EUROPE

IMPSN
International MPS Network
Mucopolysaccharide and Related Diseases

fundacja SMA

Industry

EUCOPE
European Confederation of Pharmaceutical Entrepreneurs AISBL

efpia

ultragenyx

AstraZeneca

Chiesi

sanofi

DENALI THERAPEUTICS

ACADIA®

BIOMARIN®

EVERSANA®

HLM RARE 2025: THE HOSTS AND ERN COORDINATORS LEADING THE MEETING









In order of appearance: 1. HLM Rare 2025 – room at the Residence Palace; 2. HLM Rare 2025 – room at the European Parliament; 3. & 4. Maurizio Scarpa and MEP Vytenis Andriukaitis, hosts of the HLM Rare 2025, opening the meeting; 5. Ruth Ladenstein, PaedCan Coordinator, 6. Holm Graessner, ERN-RND Coordinator; 7. Mar Manu Pereira, ERN-EuroBloodNet Coordinator; 8. Luca Sangiorgi, ERN-BOND Coordinator; 8. Alexis Arzimanoglou, EpiCare Coordinator

HLM RARE 2025: SOME OF THE HIGH LEVEL POLICY-MAKERS






In order of appearance: 1. Ursula von der Leyen, European Commission President; 2. Roberta Metsola, President of European Parliament; 3. António Costa, President of the European Council; 4. Marija Jakubauskienė, Lithuanian Health Minister; 5. Olivér Várhelyi, European Commissioner for Health and Animal Welfare; 6. Ekaterina Zaharieva, Commissioner for Startups, Research and Innovation; 7. Teresa Ribera (Executive Vice-President for a Clean, Just and Competitive Transition; 8. Emer Cooke, European Medicines Agency, Executive Director

HLM 4RARE

In 2026, HLM Rare is evolving into the Health Leadership Mission for Rare Diseases (HLM4RARE), a strategic, mission-oriented forum designed to move from political alignment to coordinated delivery.

New website: www.hlm4rare.eu

OBJECTIVES OF HLM4RARE

In 2026, the HLM4Rare will aim to:	
Main objectives	**Targets**
• **Garner community support for the Declaration**, including through a growing number of official endorsing organisations as well as individuals	• Growing the number of endorsing organizations and individuals (now more than 2000 signatures)
• **Convene multi-stakeholder Working Groups** that will come together to develop targeted Plans of Action to achieve the ambitions set out by each of the recommendations outlined in the Declaration.	• Convening 8 WG, ensuring the involvement of 3-5 EU Institutions or MS representatives across the different WG as well key advocacy stakeholders that focus on more common diseases
• **Launch targeted Plans of Action** at launch at HLM4Rare 2026 meeting	• Developing targeted Plans of Action (roadmaps)

GOVERNANCE

Core Team (CT)

- Maurizio Scarpa
- Ruth Ladenstein
- Mar Manu Pereira
- Holm Graessner
- Alexis Arzimanoglou
- Luca Sangiorgi
- MEP Vytenis Andriukaitis

- Shaping the HLM4Rare 2026 conference programme and preparatory activities based on the strategic direction provided by the SC.
- Overseeing day-to-day implementation of the work plan by the HLM Rare secretariat.

Steering Committee

- ERN Coordinators
- EURORDIS
- ERNs Patient Advocates Delegation
- FESCA
- Children's Tumour Foundation
- Rare Disease International
- International MPS and Related Diseases Network
- European Business Summit
- EUCOPE
- International Society for Neonatal Screening
- Innovation for Global Health Institute
- IPOPI
- EFPIA
- Other partners & sponsors

- Provides strategic direction to CT, helping define priorities, themes and agendas
- Participates in Working Groups and HLM4Rare Activities (e.g., policy briefings)

Working Groups

- **8 Working Groups**

- Implement the Declaration
- Support Core Team with the development of Plans of Action for implementing the Declaration

ABOUT THE WORKING GROUPS (WG)

Why	**To develop 8 targeted Plans of Action** to achieve the ambitions defined by the recommendations set out by the Declaration launched at HLM Rare. Each Plan of Action will be dedicated to one recommendation set out by the Declaration
Co-Chairs	**2 ERN Coordinators per WG** whose responsibility will be to A) co-lead the development of the targeted Plan of Action, B) co-chair WG meetings, and C) co-lead on the preparatory work needed to deliver on A and B
Members	**Circa 15-20 stakeholders in each WG** including representatives from patient organisations, ERNs, medical societies, industry, EU Institutions, and Member States

HLM4RARE WORKING GROUPS: STATUS

WG1: Action Plan

Chairs: Alexis Arzimanoglou; Maurizio Scarpa

Declaration: Prioritise the EU Action Plan on rare diseases with clear governance unifying the rare disease community.

Status/focus: meeting to be scheduled; will build on outputs from other WGs.

WG2: Workforce development

Chairs: Birutė Tumienė; Teresinha Evangelista

Declaration: Strengthen workforce development and capacity building for rare diseases and formalise the ERN Academy.

Status/focus: first meeting held; strategic priorities survey launched.

WG3: Equitable access to diagnosis and care

Chairs: Maurizio Scarpa; Holm Graessner

Declaration: Accelerate equitable access to diagnostics and enable early treatment onset with innovative orphan drugs and digital innovation.

Status/focus: two pillars agreed: newborn screening and rapid genetic diagnosis.

WG4: Clinical trials

Chairs: Holm Graessner; Luca Sangiorgi

Declaration: Foster EU leadership in rare disease clinical trials through inclusive collaboration between academia, ERNs, patients and industry.

Status/focus: four priorities agreed; members moving into subgroups.

WG5: CoRDICs

Chairs: Ruth Ladenstein; Holm Graessner

Declaration: Create, in each Member State, at least one comprehensive rare disease infrastructure cluster for research, innovation and care.

Status/focus: CoRDIC concept discussed; next step: scope and implementation strategy.

WG6: RWE and data

Chairs: María del Mar Manu Pereira; Franz Shaefer

Declaration: Boost real-world evidence by ensuring rare-disease data are high-quality, interoperable and clinically useful within EHDS and AI Act.

Status/focus: 17 actions mapped across four priorities; survey feedback discussed.

WG7: Early access models

Chairs: Maurizio Scarpa; Alexis Arzimanoglou

Declaration: Explore early access models and mechanisms to prioritise equitable access to innovative orphan therapies and diagnostics.

Status/focus: meeting in preparation; focus on JCA experience and orphan medicines.

WG8: Multiannual Financial Framework

Chairs: MEP Vytenis Andriukaitis; Maurizio Scarpa

Declaration: Ringfence European Reference Networks funding under the 2028–2034 Multiannual Financial Framework.

Status/focus: input feeding Biotech Act amendments and ERN value recognition.

THE 8 WORKING GROUPS (WG)

WG 1: Action Plan for Rare Diseases

ERN Coordinators Leads: Alexis Arzimanoglou and Maurizio Scarpa
**Across topics*

WG 2: Workforce development

ERN Coordinators Leads: Birutė Tumienė and Theresinha Evangelista

WG 3: Equitable access to diagnosis and care

ERN Coordinators Leads: Maurizio Scarpa and Holm Graessner

WG 4: clinical trials

ERN Coordinators Leads: Holm Graessner and Luca Sangiiorgi

WG 5: Clusters for research, innovation and care (CoRDICs)

ERN Coordinators Leads: Ruth Ladenstein and Holm Graessner

WG 6: Real-world evidence

ERN Coordinators Leads: María del Mar Mañú Pereira and Franz Shaefer

WG 7: Early access models

ERN Coordinators Leads: Alexis Arzimanoglou and Maurizio Scarpa

WG 8: Multiannual Financial Framework*

Coordinator: MEP Vytenis Andriukaitis (S&D, LT)
**Across topics*

OUR 3 KEY EXTERNAL MILESTONES

Rare Disease Day
28 February

ERN Meeting (led by the EU Commission)
26 March, Brussels

European Conference on Rare Diseases (ECRD) 2026
3-4 June, Prague

Conference on Rare Diseases by the Cyprus Presidency of the Council of EU
16-17 June, Nicosia

'Health without Postcodes' European Parliament event
1 July, Brussels

This event will focus on Joint Clinical Assessments, early access models and the broader coordination needed to accelerate equitable and timely access to therapies for people living with rare and complex diseases.

European Health Forum Gastein session
29 September - 2 October, Bad Hofgastein (Austria)

This session will bring HLM4Rare to a wider policy audience beyond the rare and complex diseases community.

World Orphan Drug Congress Europe
26-28 October, Barcelona

HLM4Rare 2026 meeting
1 – 3, December, Brussels

Timelines & next steps towards HLM4Rare 2026

1st meeting
24th April 3-5 pm

Objectives

- Explain the methodology and division of responsibilities for drafting.
- Align on success criteria of the Plan of Action.
- Map the problem, context, EU instruments, decision windows and stakeholders relevant to the policy recommendation.
- Agree on 3–5 priority areas/themes the Plan of Action must cover.

2nd meeting
1st June 3-5 pm

Objectives

- Define clear goals per priority area, with high-level indicators of success.
- Identify strategies and major actions under each goal.

3rd meeting
September/October

Objectives

- Review and refine the draft Plan of Action.
- Complete action details (responsible, timeline, resources, monitoring).
- Secure agreement on the plan and define follow-up governance

4th meeting
November/December

Objectives

- Discuss relevant feedback shared during the HLM4Rare conference and any final changes needed on the Plan of Action.
- Kick-off discussions on next steps for implementation.

Current Working Group status:

- **WG1:** Set-up / scheduling
- **WG2:** 1st meeting & inputs
- **WG3:** Priorities agreed
- **WG4:** Priorities agreed
- **WG5:** 1st meeting & inputs
- **WG6:** Action plan drafting
- **WG7:** Set-up / scheduling
- **WG8:** Policy uptake / advocacy

EUROPEAN JOINT ACTIONS ON CANCER

European Joint Actions on Cancer

EU Join Actions on Cancer

EU JOINT ACTION JANE2 NETWORKS OF EXPERTISE ON CANCER

EUnetCCC ONEFORALL Co-funded by the European Union

JA PCM Joint Action Personalised Cancer Medicine

JA PreventNCD

eCAN Enhancing digital capabilities of cancer centres in Europe

EU CanScreen European Joint Action on Cancer Screening

EU Cancer Mission

Rare Cancer Forum

ERNs on rare cancers

ern eurobloodnet ERN | EuroBloodNet

EURACAN

European Reference Network for rare or low prevalence complex diseases

Network Paediatric Cancer (ERN PaedCan)

ERN GENTURIS With every diagnosis we can help an entire family

European Associations on Cancer

eha

ESMO

EORTC European Organisation for Research and Treatment of Cancer

esso

SIOPE SIOP Europe the European Society for Paediatric Oncology

JANE-2

Joint Action coordinated by Fondazione IRCCS Istituto Nazionale dei Tumori (INT), Italy.

- Paolo Casali

There are 29 Beneficiaries, 92 Affiliated Entities and 3 Associated Partners across Europe.

- 121 organizations from 29 European countries.

ERNs: PaedCan, EURACAN, ERN Genturis, **ERN-EuroBloodNet.**

Goal: improve cooperation on the 7 NoEs.

Based on: **Rare Cancer Agenda 2030**

7 new Networks of Expertise (NoE / WPs)

1. **NoE on complex and poor-prognosis cancer(s)**
2. **NoE on palliative care**
3. **NoE on survivorship**
4. **NoE on personalized primary prevention**
5. **NoE on omic technologies**
6. **NoE on hi-tech medical resources**
7. **NoE on adolescents and young adults with cancer**

RARE CANCER FORUM

- Launched in summer 2025 under the umbrela of EU Cancer Mission.
- Chaired by members of the Cancer Mission Board, namely Prof. Christine Chomienne, Dr. Ben Westphalen and Dr Amanda Drury.
- Goal: to create a dedicated **multi-stakeholder platform** to facilitate structured dialogue, collaboration, and **alignment in addressing the unmet needs of rare cancer patients** and their caregivers across Europe.

4 WGs

1. **Tracking and treating (EORTC)**
2. **Education and Training (ESSO)**
3. **Clinical Practice Guidelines (ESMO)**
4. **Access to diagnostics and care (EHA)**

Rare cancer forum (lugano, 11.03.26), aligned with the HLM working groups.

RARE CANCER FORUM	HLM4RARE WORKING GROUP
Proposal for a European Rare Cancer Hub	**WG 1**: Governance & Action Plan
Regulatory science and policy engagement	**WG 2:** Workforce Development
Coordination with National & European Structures	**WG 5:** CoRDIC: National Infrastructure
Implementation Science and Translating knowledge into practice	**WG 2:** Workforce Development
Education, Training and Capacity Building	**WG 2:** Workforce Development
Clinical Research and Access to Clinical Trials	**WG3:** Diagnostics & Early treatment. **WG4/WG7:** Clinical Trials/Equitable access to therapies
Data Infrastructure and Registries	**WG 6**: Real-World Evidence
Stakeholder Engagement and Patient Involvement	**WG 1:** Governance & Action Plan

ERN EHA COLLABORATION

Name	Affiliation
Robin Doeswijk	EHA (Head of European Affairs)
Ariane Weinman	Eurordis (Public Affairs)
Elizabeth Macintyre	EHA (Diagnostic)
Antonio Almeida	EHA (CTs and Education)
Sally Batten	EHA (Scientific and Regulatory Relations)
Alice Di Giulio	EHA (Policy Officer)
Gauthier Quinonez	EHA (Policy Officer)
Mar Mañú Pereira	ERN-EuroBloodNet (Scientific Coordinator, EHA European Affairs Committee)
Matteo Della Porta	ERN-EuroBloodNet; IRCCS Clinical Institute Humanitas – Rozzano (Expert in Myeloid)
Luca Malcovati	ERN-EuroBloodNet; Foundation IRCCS Polyclinic San Matteo, Pavia (Expert in Myeloid)
Natacha Bolaños	ERN-EuroBloodNet; ePAG – Lymphoma Coalition
Alba Albert Robledo	ERN-EuroBloodNet (Scientific Manager)

10 YEARS OF ACHIEVEMENTS since 2017

Connecting expertise. Advancing knowledge. Improving lives across Europe.

ERN | EuroBloodNet

CROSS BORDER HEALTH

>900 RHD experts

10 ePAGs

8 European mapping exercises

27 RHD patients assisted
11 Ukranian

53 Patient Organizations

BEST PRACTICES

72 RHD CPGs & CDSTs adopted by the ERN

4 CDSTs developed: Sickle Cell Disease (x2), Pyruvate Kinase Deficiency, Methemoglobinemia.

3 CPGs developed: Pyruvate Kinase Deficiency, Burkitt lymphoma, VEXAS Syndrome.

51.22% Use of Orphacodes

CONTINUING MEDICAL EDUCATION

6 European Collaborative programs with Genomed4All, SYNTHEMA, EDITSCD, EHC, EHA, ISLH

4 preceptorships programs

515 Accredited activities

245 Non-accredited activities

EDU-Blood Academy **>370** videos

TELEMEDICINE

80 experts registered in CPMS

48 cases, **21** closed with outcome report.

61 patients enrolled

CLINICAL TRIALS AND RESEARCH

677 CTs

2 Academic Clinical Trials

90 observational studies

37 Publications

6600+ ENROL registry
5 RHD areas aligned

11 EU-Funded projects

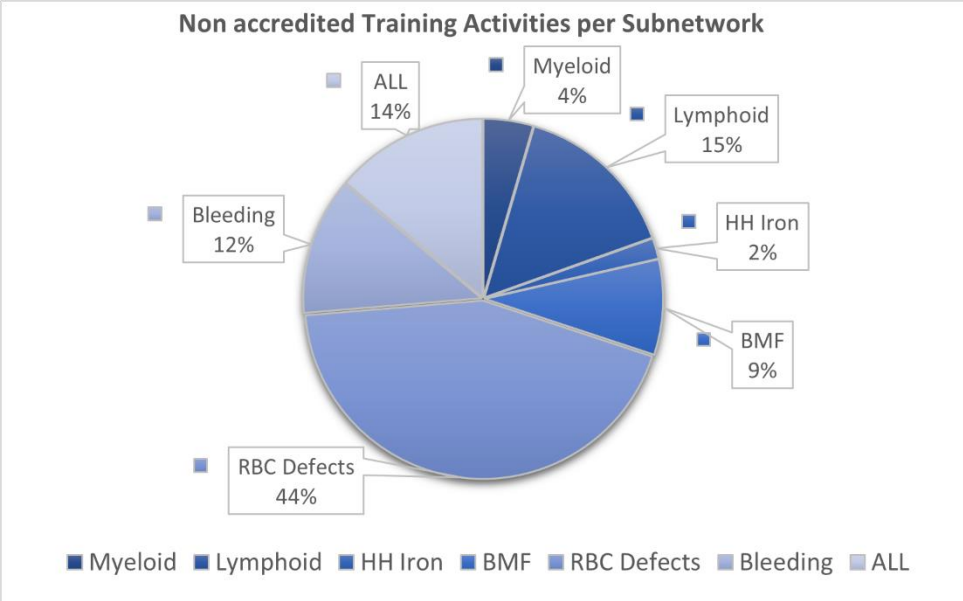
A DECADE OF COLLABORATION, INNOVATION AND BETTER CARE FOR PATIENTS

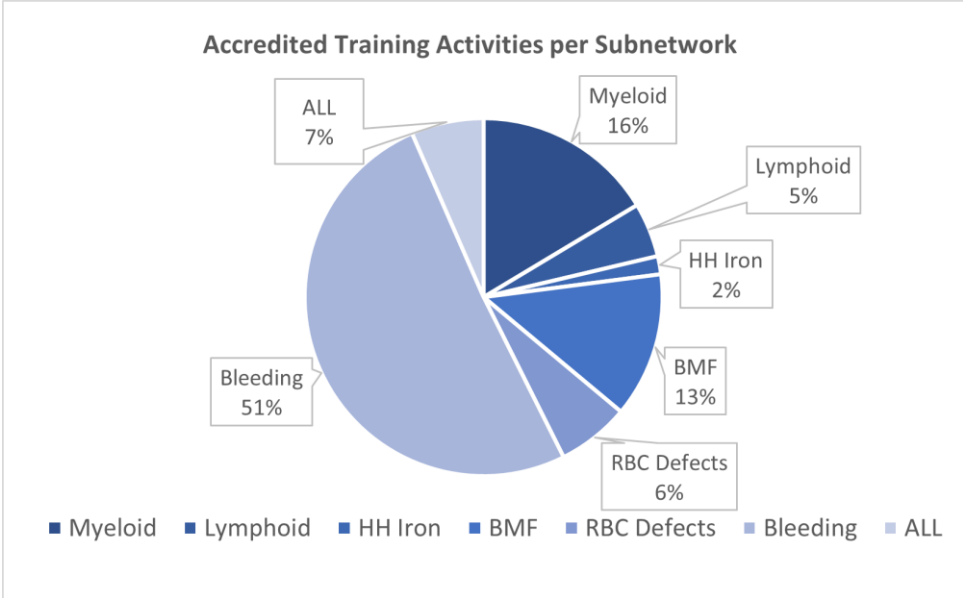
Educational activities Oct 2023-Dec 2025

Connecting expertise. Advancing knowledge. Improving lives across Europe.

ERN | EuroBloodNet

CONTINUING MEDICAL EDUCATION





Subnetwork	N° of activities
Myeloid	12
Lymphoid	40
RBC Defects	116
BMF	23
Bleeding	33
HH Iron	5
ALL Diseases	37

Subnetwork	N° of activities
Myeloid	10
Lymphoid	3
RBC Defects	4
BMF	8
Bleeding	31
HH Iron	1
ALL Diseases	4

A DECADE OF COLLABORATION, INNOVATION AND BETTER CARE FOR PATIENTS

10 YEARS OF ACHIEVEMENTS

Connecting expertise. Advancing knowledge. Improving lives across Europe.

ERN | EuroBloodNet

COLLABORATIVE PROJECTS

ERICA

ERDERA
European Rare Diseases Research Alliance

Together4RD

Realise D
compRehensive mEthodological and operational Approach to cLinical trIalS in ultra-rarE Diseases

GENOMED 4ALL

SYNTHEMA

HemaFAIR

IMPACT - AML
European project on Relapsed/Refractory Acute Myeloid Leukemia

EDITSCD

HELIOS

EVIDENCE

INHERENT
International Hemoglobinopathy Research Network

A DECADE OF COLLABORATION, INNOVATION AND BETTER CARE FOR PATIENTS

ERN-EuroBloodNet TFAs & HLM4Rare

HLM4RARE WORKING GROUP	ERN-EUROBLOODNET TFAS
WG 1: Governance & Action Plan	
WG 2: Workforce Development	 Continuing Medical Education & Best Practices
WG3: Diagnostics & Early treatment.	 Cross border health & Clinical Trials & Telemedicine
WG 4: Clinical Trials	 Clinical Trials & Research
WG 5: CoRDIC: National Infrastructure	 Cross Border Health & Best Practices
WG 6: Real-World Evidence	 Clinical Trials & Research
WG7: Equitable access to therapies	 Clinical Trials & Research
WG 8: Funding & Sustainability	

THANK YOU!

ERN | EuroBloodNet

RADeep

www.eurobloodnet.eu

@ERNEuroBloodNet

eurobloodnet-european-reference-network-on-rare-hematological-diseases

Eurobloodnet - European Reference Network on Rare Hematological Diseases

ERN-EuroBloodNet's EDUcational Youtube channel

www.radeepnetwork.eu

European Reference Network | Hematological Diseases (ERN EuroBloodNet)

Funded by the European Union. Views and opinions expressed are however those of the author(s) only and do not necessarily reflect those of the European Union or European Health and Digital Executive Agency (HaDEA). Neither the European Union nor the granting authority can be held responsible for them.

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ERN | EuroBloodNet

12th June 2026 - EHA Congress

Patient perspective

Speaker: Angelo Loris Brunetta

European Reference Network | Hematological Diseases (ERN EuroBloodNet)

Funded by the European Union

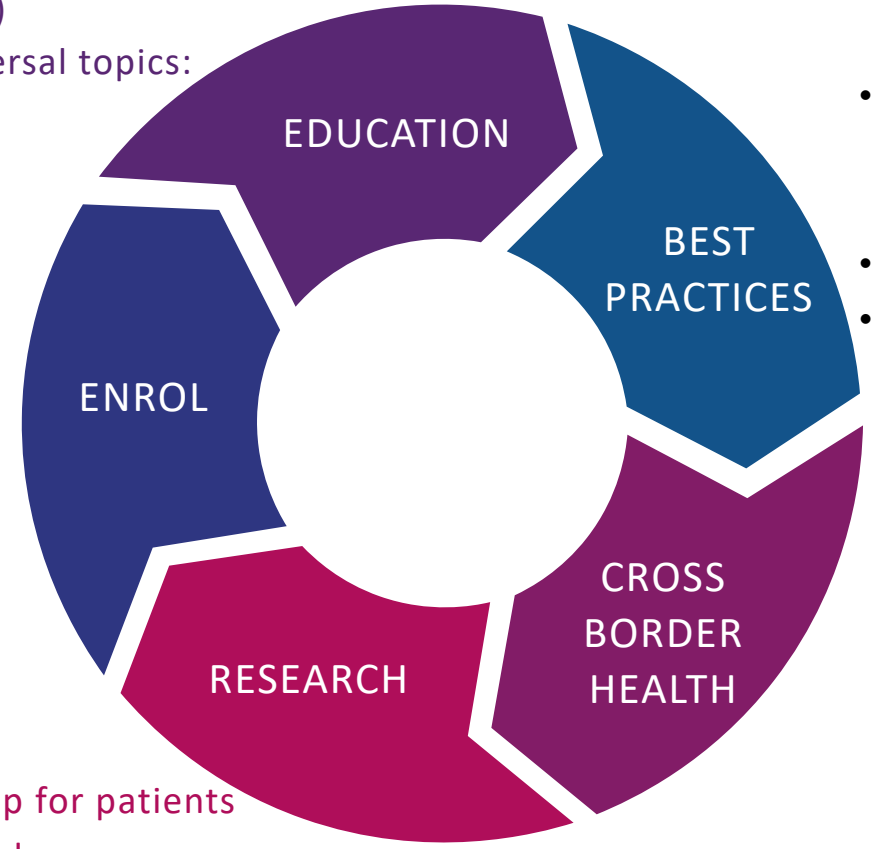
Disclosure of Conflict of Interest

"The speaker declares no conflicts of interest."

What we've done

Online & Onsite trainings (**53 webinars and on-site, 163 video**)

- Patient therapeutic education (cutaneous lymphoma, sickle cell disease (SCD), Congenital dyserythropoietic anemia, myelodysplastic syndromes, von willebrand disease (vWD))
- Advocacy trainings (SCD)
- PPI trainings (SCD)
- TiF (**MDS, SCD, PNH and AA, TTP**)
- Research Trainings (SCD + Transversal topics: Artificial intelligence, REGISTRY)



- ENROL Inform consent
- ENROL Policy of access
- ENROL WEBINAR

Engagement:

- Data Access Committee
- Contribution to Informed consent and policy of access
- Teaching in webinars

- Mapping of available drugs (Paroxysmal nocturnal hemoglobinuria, myeloproliferative neoplasms)
- Guidelines development (Burkitt Lymphoma)
- Patient Journeys (vWD)

Engagement: co-cordination

- Cross Border Health (CBH) rights tool
- Assistance to patients (**48 pts + 11 Ua**)

Engagement:

- Co-design the CBH right tool
- Assisting patients dealing with CBH procedures

- RADeep
- Genomed4all e-workshop for patients
- Genomed4all educational programs

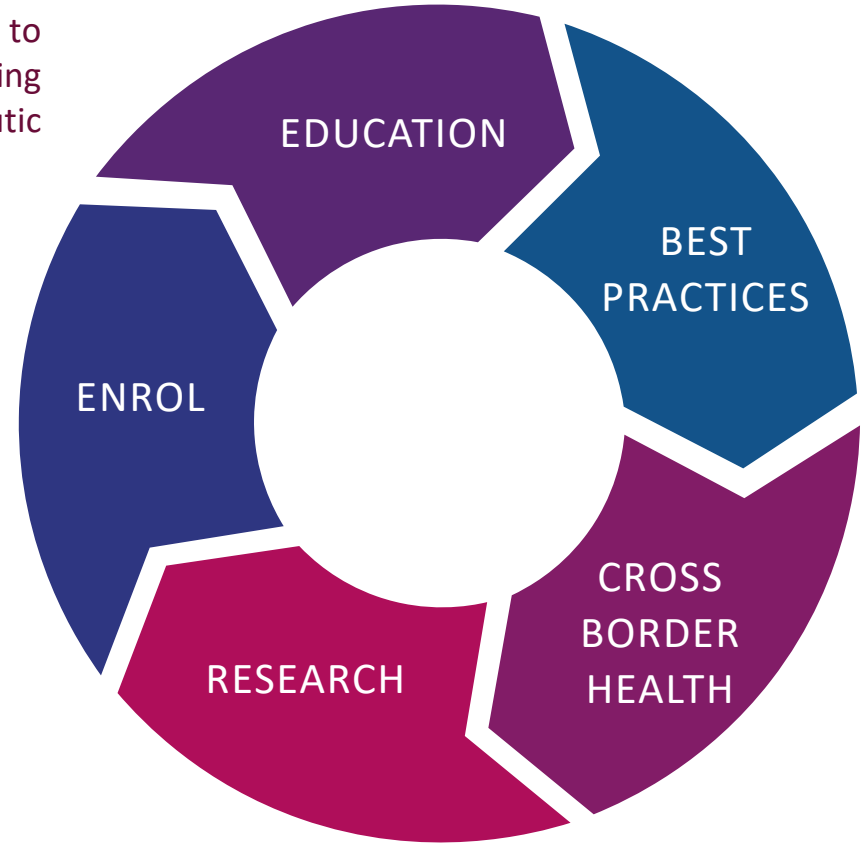
Engagement:

- Participation in the steering committee
- Co-design educational activities

What we need to do

Covering much more diseases to educate patients, increasing awareness on new therapeutic options

Collaborating both with ENROL and RADeep registries as DAC members but also to increase participation to those sites still non respondants



Strenghtening the collaboration with EHA Guidelines Committee to incentivize the production of Guidelines for ultra rare diseases, having patients involved in the process

Continuing to support patients for CBH, advancing proposal to reduce the bureaucratic burden

Stimulating research mainly on ultra-rare or neglected diseases by promoting clinical trials, incentivizing patient particpation

THE 8 WORKING GROUPS (WG)

WG 1: Prioritise the EU Action Plan on rare diseases with a clear governance unifying the rare disease community

Patient involvement helps to strenghten RD community

ERN Coordinator Lead: Alexis Arzimanoglou

WG 2: Strengthen workforce development and capacity building for rare diseases and formalise the ERN Academy

Partenring with Patient Organizations, experienced in capacity building activities

ERN Coordinator Lead: Maurizio Scarpa

WG 3: Accelerate equitable access to diagnostics and enable early treatment onset with innovative orphan drugs and digital innovation for unmet medical needs

Expert patients can support and promote early access and R&D

ERN Coordinator Lead: Luca Sangiorgi

WG 4: Foster EU leadership in clinical trials for rare diseases through inclusive collaboration between Academy, ERNs, patient groups and industry to accelerate innovation for PLWRD

ERN Coordinator Lead: Holm Graessner

WG 5: Create, in each Member State, at least one comprehensive rare disease infrastructure cluster for research, innovation and care (CoRDIC)

Partnering with Patient Organizations

ERN Coordinator Lead: Ruth Ladenstain

WG 6: Boost the generation of real-world evidence by ensuring that rare-disease data are high-quality, interoperable, and clinically useful, to accelerate biomarker discovery and support drug development in rare diseases within the EHDS and EU AI Act Regulation

ERN Coordinator Lead: Mar Manu Pereira

WG 7: Explore new early access models and mechanisms to prioritise equitable access to innovative orphan therapies and diagnostics, also supported by a European Guarantee Fund

Partnering with POs is pivotal for allowing patient access to innovation

ERN Coordinator Lead: Maurizio Scarpa

WG 8: Ringfence European Reference Networks funding under the 2028-2034 Multiannual Financial Framework

POs can support political efforts using their lobby's capacities

ERN Coordinator Lead: MEP Vitenys Andriukaitis (S&D, LT)

Funded by the European Union

Steps toward the future

Through these two Addendum issued by the EC in 2014, the ERNs have been strongly invited to bring on board patient advocates belonging to umbrella european organizations or even local or eventually patient support groups where organizations don't exist.

17.5.2014 EN Official Journal of the European Union L 147/71

COMMISSION DELEGATED DECISION

of 10 March 2014

setting out criteria and conditions that European Reference Networks and healthcare providers wishing to join a European Reference Network must fulfil

(Text with EEA relevance)

(2014/286/EU)

The application was not mandatory therefore the ERNs behaved differently, according to capacity of any single organization to struggle for having their representatives involved

17.5.2014 EN Official Journal of the European Union L 147/79

COMMISSION IMPLEMENTING DECISION

of 10 March 2014

setting out criteria for establishing and evaluating European Reference Networks and their Members and for facilitating the exchange of information and expertise on establishing and evaluating such Networks

(Text with EEA relevance)

(2014/287/EU)

The role of patients and their organizations **have to be officially recognized** as full members of the ERNs

Joint effort is not enough

European Patient Advocacy Group

17th September 2025

A joint call to formalise and strengthen patient representation in the European Reference Networks

Dear Prof. Ruth Ladenstein, Dr. Mar Mañu, and Dr. Luca Sangiorgi,

We, the undersigned representing xx (number) European Patient Advocacy Groups and EURORDIS, **call on all 24 Coordinators of the European Reference Networks (ERNs) to engage with us in a dialogue to find the best way to strengthen the recognition and institutionalise the role of patient organisations and patient representatives in the ERN system as a whole and across each Network**.

The Lead-Up

Ten years ago, the European Union Committee of Experts on Rare Diseases (EUCERD) formally recognised the need to involve patient representatives as formal members of the decision-making structures of the European Reference Networks[1].

People living with a rare or complex condition draw on their lived experience, and some also bring knowledge acquired through training, complementing the scientific expertise of health professionals and building together a critical mass of knowledge and expertise to address some of the most pressing needs of this patient population. Given the specificity of rare and complex conditions, the EUCERD concluded that a high level of involvement of patient representatives in decision-making processes was essential to ensure the successful development of the Networks.

Specifically, the EUCERD recommended that ERNs demonstrate meaningful patient involvement and patient-centredness by recognising the role of people living with rare and complex conditions as experts by experience and co producers of knowledge in the ERNs' structural and clinical activities.

By the time the Networks were launched in 2017, the European Commission had established the rules and criteria for the participation of healthcare providers in the 24 Networks[2]. However, the legislation did not develop the mechanisms to formalise the involvement of patient organisations and patient representatives in the ERN system, nor in the individual ERNs. This legislative vacuum in the ERNs system, contrasts with the recent HTA Regulation (EU) 2021/2282, which embeds the roles of patient representatives and patient organisations into the HTA processes, Joint Clinical Assessments (JCAs) and Joint Scientific Consultations (JSCs), and with the EU pharmaceutical legislation, Regulation (EC) No 726/2004 and Directive 2001/83/EC, under which the EMA has institutionalised patient involvement at each stage of the medicine development lifecycle.

A few ERNs formally included patient organisations in their initial applications and governance structures, and the majority have progressively integrated patient representation into their governance structures through the

[1] European Union Committee of Experts for Rare Diseases Addendum to EUCERD Recommendations on Rare Disease European Reference Networks of January 2013, adopted in 10 June 2015.
[2] OJ L 174, 17.5.2014, p. 71, Commission Delegated Decision 2014/286/EU of 10 March 2014 setting out criteria and conditions that European Reference Networks and healthcare providers wishing to join a European Reference Network must fulfil and OJ L 174, 17.5.2014, p. 79, Commission Implementing Decision 2014/287/EU of 10 March 2014 setting out criteria for establishing and evaluating European Reference Networks and their Members and for facilitating the exchange of information and expertise on establishing and evaluating such Networks.

A letter signed from 33 ePAGs representing 20 out of 24 ERNs belonging to the Eurordis ePAGs Steering Committee, sent to the ERNs Coordinators was not considered sufficiently relevant to ask the EC to offcially recognize the patient representativeness in ERNs.

European Reference Networks

TO EURORDIS

Virginie HIVERT, Acting Chief Executive Officer

Ref: EURORDIS and the ePAG Steering Committee proposal to formalise the involvement of patient representatives and patient organisations in the ERNs and the ERNs system.

Dear EURORDIS Team,

Dear ePag Steering Committee

This is in response to your letters and to provide further clarification on the response we previously provided. Since ERNs' inception, all ERNs have included Patient Advocacy Groups in their structures. Patient representatives contribute to clinical pathway designs, guidelines development, registry governance, and research prioritization. Their role is truly operational, not symbolic. The above was confirmed by a recent survey across all ERNs. Patient Advocacy Groups presented in Eurodis, according to this survey, are at a median 47% and taking the mean 52%. This clearly shows that, although EURORDIS is an appreciated and strong voice in the Rare Disease Community, it does not represent all patient advocates, advocacy groups or Patient Associations or Federations represented within the 24 ERNs. Hence, we need to state, that Patient Advocates across ERNs are very involved and welcome within the ERNs having active roles and receive funding support through the ERNs, for example when sending a delegation to our annual meetings or to publish their Newsletters etc Therefore, all need to be equally valued and recognised.

Regarding the composition of the Board of Member States, we reiterate that its composition is defined by the existing legal framework. Any modification would require formal legislative consideration. This is unlikely to happen prior to the JARDIN project delivering, and common conclusions may be drawn according to DG SANTE.

It is therefore important to distinguish between the two topics:

First, patient participation within ERNs is robust and active, independently of the EURORDIS membership of many of them.

Second, institutional architecture at the Member State level, which is legally defined.

EURORDIS is a valued partner in the rare disease community. Its advocacy has been instrumental in shaping European rare disease policy. Dialogue continues to be active and constructive.

ePAGs

THANK YOU!

European Reference Network | Hematological Diseases (ERN EuroBloodNet)

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