



ERN-EUROBLOODNET Action Plan and Governance

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SESSION 1: EU ACTION PLAN FOR RARE DISEASES

From European Policy to ERN-EuroBloodNet Action

PRIORITY ACTION 1 & 8: EU ACTION PLAN FOR RARE DISEASES & FUNDING



EUROPEAN PRIORITY FOR RARE DISEASES

HLM4RARE WG1: Prioritize the EU Action Plan on rare diseases with a clear governance unifying the rare disease community.

HLM4RARE WG8: Ringfence ERNs funding under the 2028-2034 Multiannual Financial Framework



CHALLENGES

- Rare diseases remain fragmented across EU and national policy agendas, limiting visibility, investment and coordinated action.
- Rare diseases struggle to compete for visibility and investment against larger public health priorities.
- Translating European collaboration into measurable patient benefit remains challenging without shared governance, indicators and targets.



ERN-EUROBLOODNET RESPONSE



Cross-border health



Best Practices



Continuing Medical Education



Telemedicine



Clinical Trials & Research

EU ACTION PLAN FOR RARE DISEASES

WG1

Governance & Action Plan

WG2

Workforce Development

WG3

Diagnostics & Early treatment

WG4

Clinical Trials

WG5

National Infrastructure

WG6

Real-World Evidence

WG7

Equitable access to therapies

WG8

Funding & Sustainability

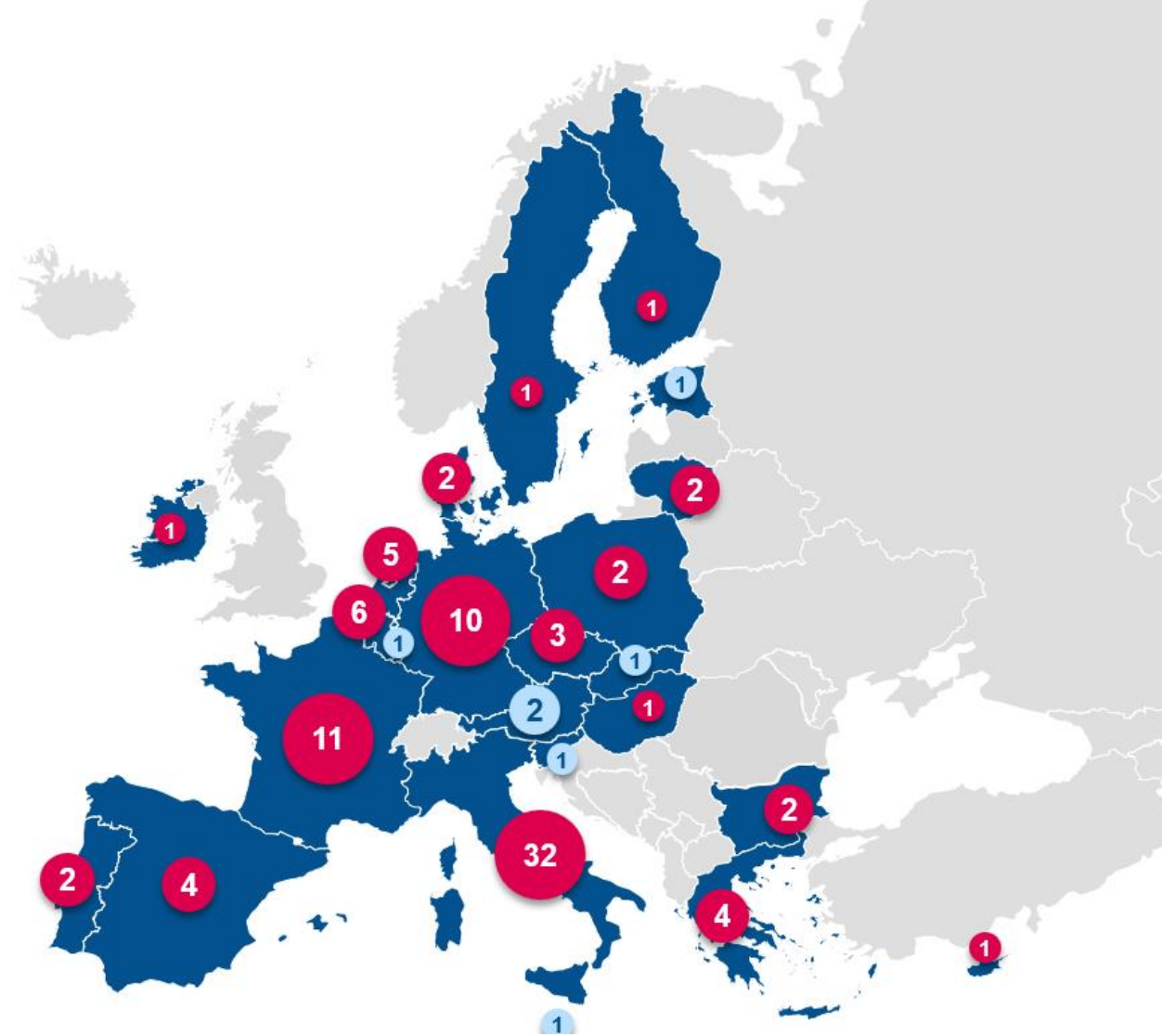




ERN-EuroBloodNet: Who we are

ERN-EuroBloodNet is a collaborative network aiming to **improve the healthcare and innovation in rare hematological diseases (RHD)**

The Board of the Network (BoN) comprises **96 Health Care Providers from 24 EU Member States** and is supported by **10 European Patient Advocacy Groups**:



4 EU Member States not covered: Croatia, Latvia, Norway and Rumania



ERN-EuroBloodNet in the **ERNs-Transversal WGs**

Transversal Working Group	Abbreviation	Associated Activities / Topics	ERN-EuroBloodNet Presence	
Knowledge Generation and Capacity	KG	Erasmus+ Program & ERNs		
		Academic specialization courses on rare diseases (RDs)		Béatrice Gulbis
		ERN Academy		Béatrice Gulbis
		Artificial Intelligence & rare diseases		Mar Mañú
		Newborn screening (NEW!)		Mar Mañú
Ethical, Legal and Social Implications	ELSI	Ethical, legal, and social aspects of ERN activities and rare disease initiatives		
Management Activities	MA	Registries		Mar Mañú, Sara Reidel
		Communications		Daiana Lopez
		Clinical Patient Management System (CPMS)		Kathleen Weatherly
Improvement of the Evaluation	EI	Evaluation methodologies, performance improvement, and assessment activities		Béatrice Gulbis
Monitoring	MO	Monitoring progress, outcomes, indicators, and implementation activities		Mar Mañú, Alba Albert, Mireia Arnedo
Transition	TR	Transition-related activities and continuity of care processes		Anna Collado, Raffaella Colombatti



ERN-EuroBloodNet: WorkPackages

TFAs
Coordination



Data-Driven and Clinical trials

Strengthening the re-use of **RHD data** for the provision of best healthcare and promotion of research



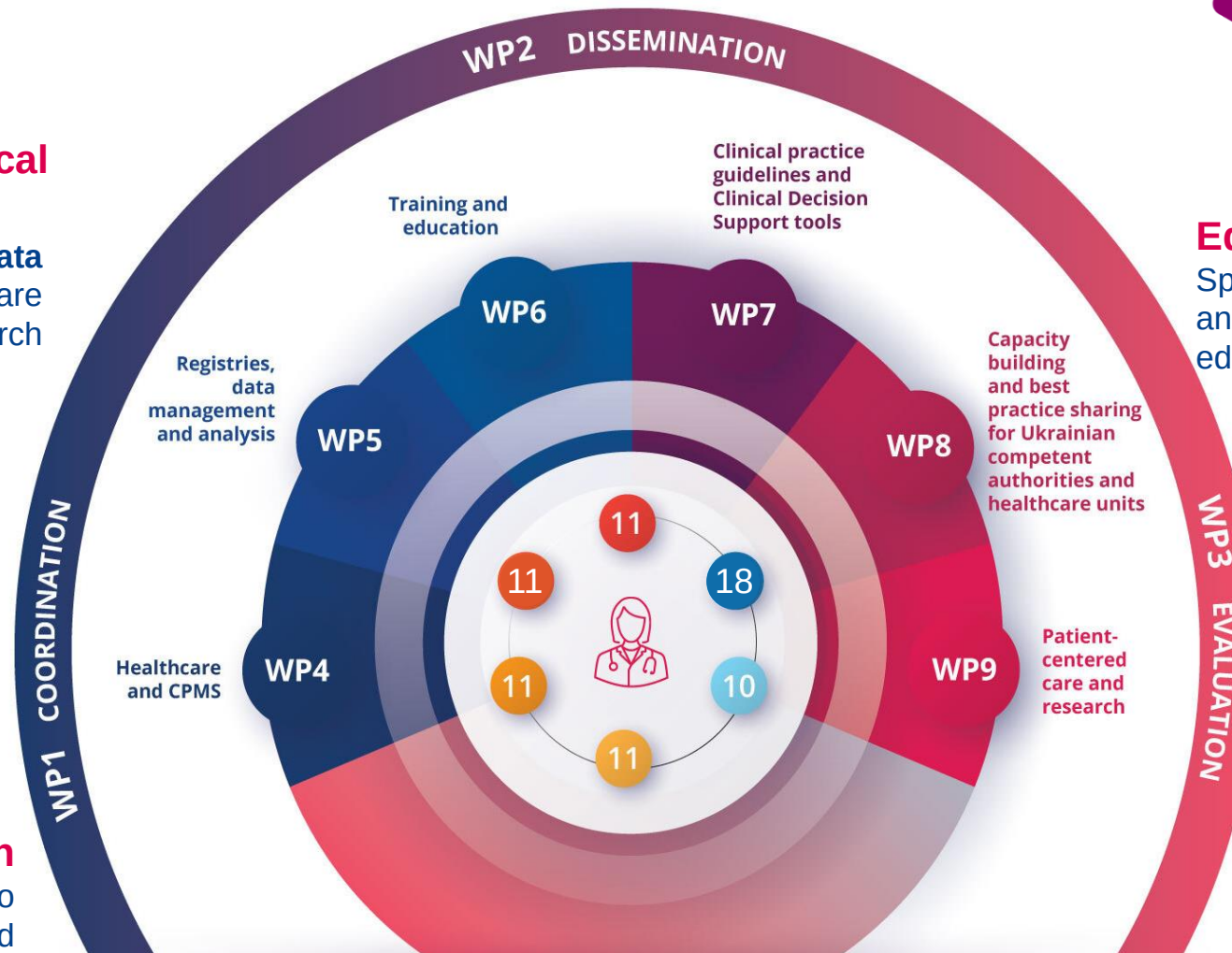
Telemedicine

Providing inter-professional **consultation for complex RHD cases** through CPMS



Cross Border Health

Advancing **equal access** to specialized, outcome-based and **cross-border RHD care** across EU.



Education and training

Spreading **cutting-edge knowledge** and facilitate continuing medical education in the field of RHDs.



Best practices

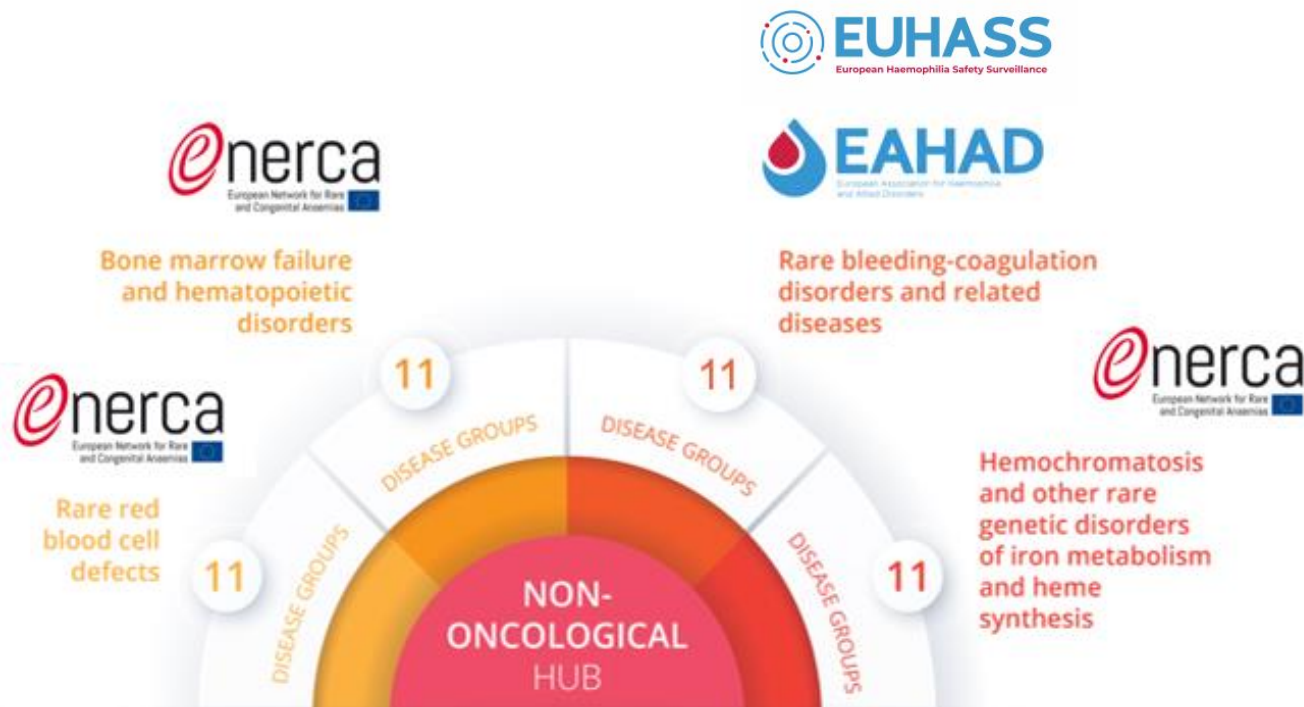
Promoting best practice in **prevention, diagnosis and care** through updated guidelines and clinical decision tools.





ERN-EuroBloodNet: Disease Coverage

ORPHA codes: 422 Disorder (661 codes) RHD + 15 Disorder (18 codes) Disorder of iron metabolism and transport
Non-oncological 253 Disorder (391 codes): adult and pediatric and Oncological 184 Disorder (287 codes): adult
6 subnetworks and 72 disease groups (Disorders and group of disorder) –expert based grouping



CLÍNICA
BARCELONA
Hospital Universitari

EuroBloodNet proposal in 2016
Spanish CSUR in hematology not yet in place in 2017 (only BMT)





ERN Coordination

3 Hubs of Coordination

- **AP-HP H St Louis:** Pierre Fenaux
Coordinator
Onco chair
- **ULB-ERASME:** Béatrice Gulbis
Co-Coordinator
Non-Onco chair
- **VALL D'HEBRON:** Maria del Mar Mañú Pereira
Scientific Coordination
ENROL Registry Coordinator



01 Board of the Network

ERN Members
Healthcare providers members of the ERN,
including one representative, one Substitute
representative

ERN Affiliated Partners
Associated National Centres and National
Coordination Hubs (no voting rights)

**European Patients Advocacy
Groups (ePAGs)**

02 Scientific & Strategic Board

Transversal Fields of Action Representatives
2 Health professionals non-Oncological and Oncological
1 ePAG

Subnetwork Representatives
2 Health Professionals
1 ePAG per subnetwork

Leaders of ongoing actions
Health professionals and ePAGs



Disease Subnetworks Representatives

Subnetwork	Representative	HCP / Association
Myeloid	Pierre Fenaux	Assistance Publique-Hôpitaux de Paris, Hôpital Saint-Louis
	Uwe Platzbecker	Universitätsklinikum Carl Gustav Carus
	Sophie Wintrich	ePAG - MDS UK Patient Support Group (and MDS Alliance)
Lymphoid	Andreas Engert	Universitätsklinikum Köln
	Catherine Thieblemont	Assistance Publique-Hôpitaux de Paris, Hôpital Saint-Louis
	Pierre Aumont	ePAG - Ensemble Leucémie Lymphomes Espoir (ELLyE) & Chronic Lymphocytic Leukemia Advocates Network (CLLAN)
RBC	Domenica Cappellini	Foundation IRCCS CA'Granda Ospedale Maggiore polyclinic - Milan
	Béatrice Gulbis	CUB-Hôpital Erasme
	Angelo Loris Brunetta	ePAG - Thalassaemia International Federation - Associazione Ligure Talassemici Onlus
BMF	Achille Iolascon	AOU Federico II - Naples
	Régis Peffault de Latour	Assistance Publique-Hôpitaux de Paris, Hôpital Saint-Louis
	Maria Piggin	ePAG - Paroxysmal nocturnal hemoglobinuria (PNH) support
Bleeding-Coag	Flora Peyvandi	Foundation IRCCS CA'Granda Ospedale Maggiore polyclinic - Milan
	Sophie Susen	CHU de Lille
	Ioana Todorova-Yanakieva	ePAG - Bulgarian TTP Patient organization
HH-Iron	Domenico Girelli	AOUI di Verona
	Dorine Swinkels	Radboud University Medical Center Nijmegen
	Dag Erling Stakvik	ePAG - European Federation of Associations of Patients with Haemochromatosis (EFAPH)



Transversal Fields of Actions Representatives

TFA	Coordination	Representative	HCP / Association
Policy, capacity building and cross border health	Oncological	Pierre Fenaux	APHP, Hôpital Saint-Louis
	Non oncological	Raffaella Colombatti	AO Padua
	ePAG	Ananda Plate	Myeloma Patients Europe
Best Practices	Oncological	Luca Malcovati	Foundation IRCCS Polyclinic San Matteo, Pavia
	Non oncological	Paola Bianchi	Foundation IRCCS CA'Granda Ospedale Maggiore polyclinic - Milan
	ePAG	Amanda Bok	European Haemophilia Consortium (EHC)
Continuing medical education	Oncological	Dominique Bron	Institut Jules Bordet
	Non oncological	Patricia Aguilar-Martinez	Montpellier University Hospital (CHU de Montpellier)
	ePAG	Jan Geissler	Leukemia Patient Advocates Foundation
CPMS	Oncological	Adèle De Masson	APHP, Hôpital Saint-Louis
	Non oncological	Béatrice Gulbis	CUB-Hôpital Erasme
	ePAG	Sophie Wintrich	MDS UK Patient Support Group (and MDS Alliance)
Clinical trials	Oncological	Pierre Fenaux	Assistance Publique-Hôpitaux de Paris, Hôpital Saint-Louis
	Non oncological	Eduard van Beers	University Medical Center Utrecht
	ePAG	Angelo Loris Brunetta	Thalassaemia International Federation - Associazione Ligure Talassemici Onlus
Research	Oncological	Matteo Della Porta	IRCCS Humanitas Research Hospital (Milan)
	Non oncological	María del Mar Mañú Pereira	Vall d'Hebron Institut de Recerca VHIR, Hospital Universitari
	ePAG	Angelo Loris Brunetta	Thalassaemia International Federation - Associazione Ligure Talassemici Onlus



10 YEARS OF ACHIEVEMENTS since 2017

Connecting expertise. Advancing knowledge. Improving lives across Europe.



CROSS BORDER HEALTH

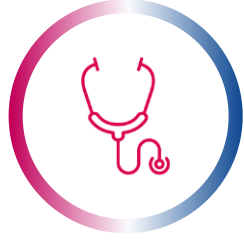
>900 RHD experts

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



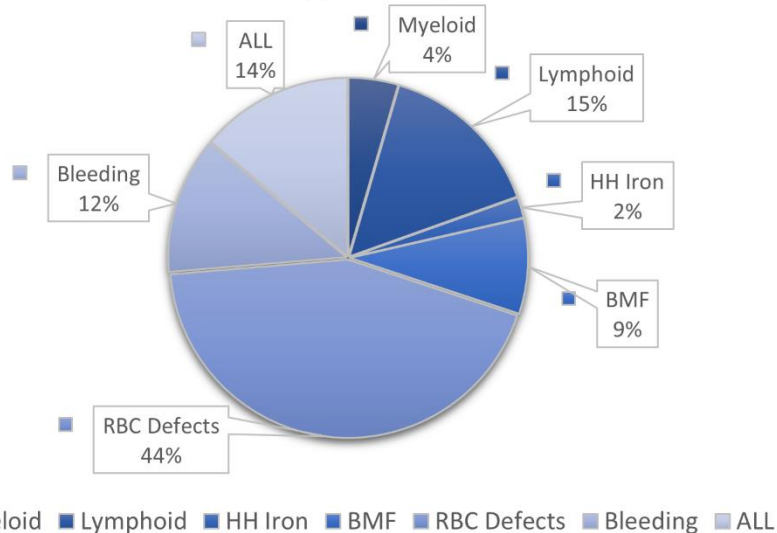
Educational activities Oct 2023-Dec 2025

Connecting expertise. Advancing knowledge. Improving lives across Europe.



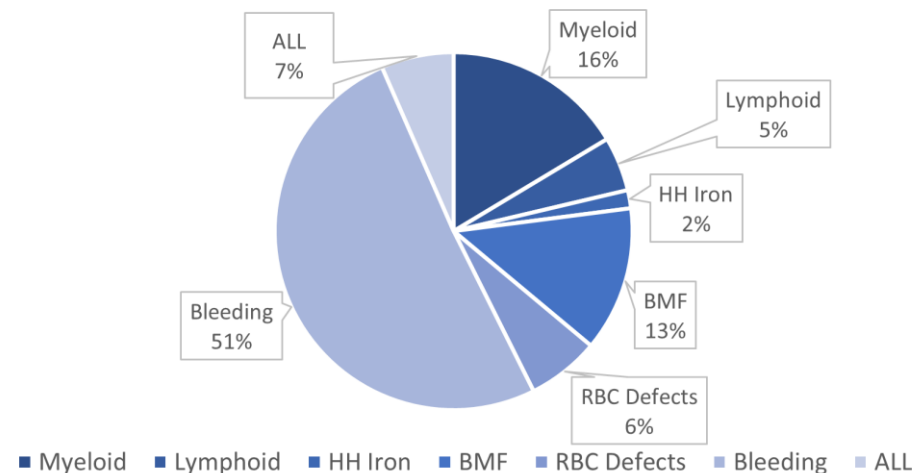
CONTINUING
MEDICAL EDUCATION

Non accredited Training Activities per Subnetwork



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

Accredited Training Activities per Subnetwork



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.

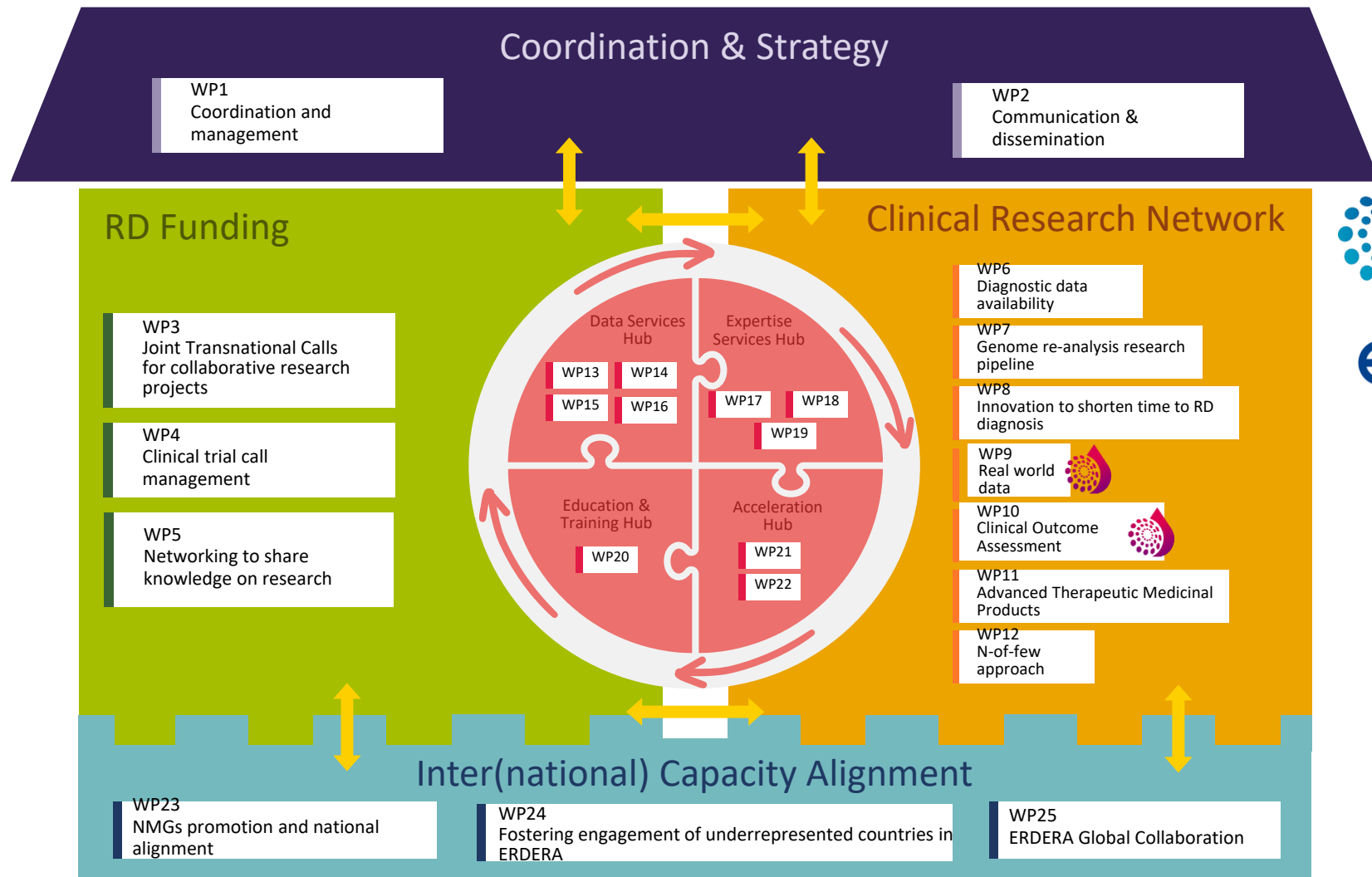


COLLABORATIVE
PROJECTS



A DECADE OF COLLABORATION,
INNOVATION AND BETTER CARE FOR PATIENTS

- Executive committee
- Outcome Research WorkStream
- WP10 Leader



WP13
Rare Diseases-Virtual Platform
(RD-VP): Finding and accessing
the data ecosystem

WP14
Data readiness services

WP15
Data sharing and analysis
services

WP16
Knowledge bases and
ontologies for RD research

WP17
Mentoring and consultancy

WP18
Regulatory support service

WP19
Methodological Support

WP20
Education and training in rare
diseases research

WP21
Technology accelerator

WP22
Public-Private Collaboration
Accelerator



Realise

D

comprehensive methodological and operational Approach to
Clinical trials in ultra-rare Diseases

VHIR, Barcelona



WP1:

T1.3 Leader (Framework for Patient Referral System)

VHIR, Barcelona



Humanitas, Milano

WP2:

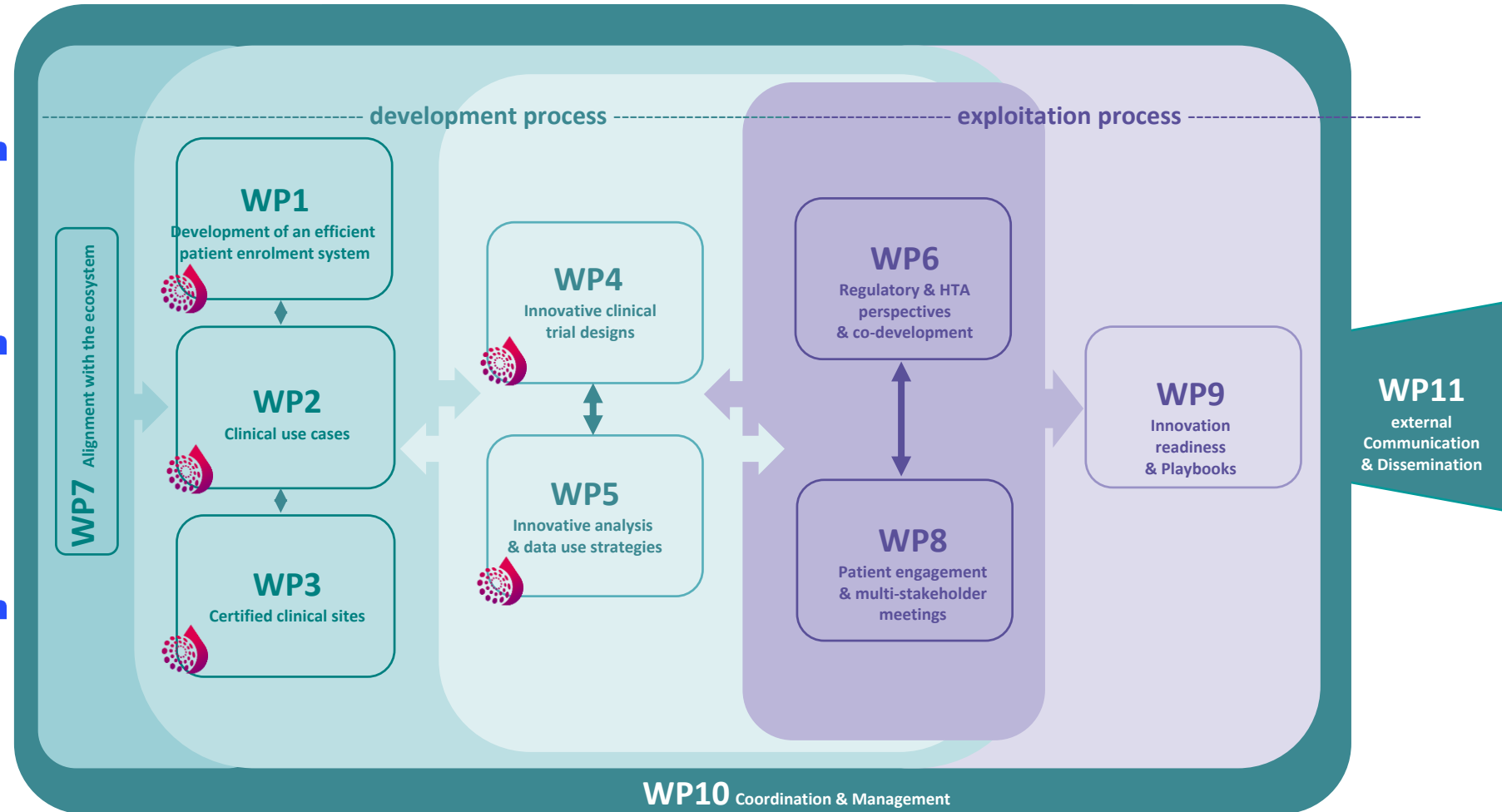
T2.1 Leader (Data readiness and management)

VHIR, Barcelona



WP3:

T3.2 co-Leader (Mapping of the EU research centres and national directives)





ERN-EuroBloodNet: Coordination



Pierre Fenaux
Coordinator
Oncological hub



Béatrice Gulbis
Co-coordinator
Non-oncological hub



María del Mar Mañú Pereira
Scientific Coordinator
ENROL Coordinator



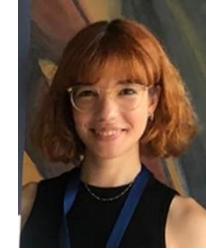
Alba Albert Robledo
Scientific manager



Mireia Arnedo
Policy Manager



Giulio Sannasardo
Project manager



Victoria Gutierrez
Scientific manager
2016-2024



Mariangela Pellegrini
Education & Patients
2017-2025



Daiana Lopez
Dissemination



Sara Reidel
Biostatistician & Data



Maria Paraskeva
Data Science



Raquel Mosull
Finances&HHRR



Patricia Gonzalez
Administration



Firdaous El Khattab
Administration&Finance



Hiba Fadlallah
Education & Patients



Sara Wambacq
Project Manager



Kathleen Weatherly
Education and CPMS



Hematological
Diseases (ERN EuroBloodNet)



Funded by
the European Union



ERN-EuroBloodNet: European Patient Advocacy Groups (ePAGS)



Marius Tanase

European Haemophilia Consortium (EHC)



Angelo Loris Brunetta

Thalassaemia International Federation



Maria Piggini

PNH Global Alliance



Ananda Plate

Myeloma Patients Europe



Natacha Bolaños

Lymphoma Coalition



Ariane Weinman
Public Affairs
senior manager



Dag Erling Stakvik

European Federation of Associations of
Patients with Haemochromatosis
(EFAPH)



Jan Geissler

Leukemia Patient Advocates Foundation



Pierre Aumont

Ensemble Leucémie Lymphomes Espoir
(ELLyE) & Chronic Lymphocytic Leukemia
Advocates Network (CLLAN)



Jana Pelouchova

MDS alliance, Diagnoza leukemie, z.s.,
Leukemia Patient Advocates Foundation,
CML Advocates Network, Chronic
Lymphocytic Leukemia Advocates
Network (CLLAN).



Ioana Todorova-Yanakieva

Bulgarian TTP Patient organization

THANK YOU!



www.eurobloodnet.eu



@ERNEuroBloodNet



[eurobloodnet-european-reference-network-on-rare-hematological-diseases](https://www.linkedin.com/company/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



Hematological
Diseases (ERN EuroBloodNet)

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