



European
Reference
Networks

ERNs State of play

María del Mar Mañú Pereira

ERN-EuroBloodNet Scientific Coordinator and ENROL Registry Coordinator

ERN CG vice-chair

Vall d'Hebron University Hospital, Barcelona

mar.manu@vhir.org



Coverage of the ERNs in the EU-MS and Norway

Clinical centres	AT	BE	BG	CY	CZ	DE	DK	EE	ES	FI	FR	GR	HR	HU	IE	IT	LT	LU	LV	MT	NL	NO	PL	PT	RO	SE	SI	SK		TOTAL	Mbrs	APs	MS not covered
BOND	1	2			1	7	3		4	2	5			1	1	11	2	1		1	4	1		2		1	1		51	44	7	9	
CRANIO	1	3			1	2	2		3	1	3			2	1	7	1	1	1	1	4	2	1	1		3	1		42	35	7	7	
Endo-ERN	2	7	2	1	2	13	3	1	5	1	9	3	2	1	1	20	1	1	2	1	9	2	4	3	4	2	1	2	105	93	12	0	
EpiCARE	2	2		1	2	4	2	2	3	2	6		2	1		7	2	1	1	1	1	1	1	3	1	1	1		50	38	12	4	
ERKNet	2	3			2	11	3	2	5	1	7	1		1	1	15	1	1	2	1	6	1	3	1	1	1	2		74	64	10	4	
ERN-EYE	3	3			1	8	2	2	7	1	6		1	1	1	9	1	1	1	1	4		1	1	1	1	1	1	59	51	8	4	
ERNICA	4	3			1	6	2	1	3	1	6		1	1		6	2	1	1	1	5	1	2			3	1		52	39	13	7	
ERN-LUNG	3	6		1	4	11	2	1	4	1	5		1	2	1	23	2	1	1	1	7	1	2	3		2	1	1	87	78	9	3	
ERN-RND	1	4	1	1	5	9	2	1	8	1	5	1	1	3	1	9	1	1	1	1	6		2			2	1		68	64	4	4	
ERN-SKIN	3	3			4	8	2		1	1	6		1	3	1	12	1	1	1	1	5				1	1	1		57	53	4	8	
EURACAN	1	6		1	4	8	1	2	6	2	13	1	2	1	1	26	2	1	1	1	9	1	3	3	1	3	1		101	92	9	2	
EuroBloodNet	2	6	2	1	3	10	2	1	4	1	11	4		1	1	33	1	1		1	5		2	2		1	1	1	97	90	7	4	
eUROGEN	1	3			2	11	2		7	1	2		1	1		7	1	1	1	1	6		2	1		4	1		56	51	5	8	
EURO-NMD	2	5	1	1	2	10	2	1	7	2	9	1		2	1	20	1	1	1	1	6	1	1	1		2	1		82	74	8	3	
GENTURIS	2	4		1	2	6	2	1	2	2	2	1		1		9	1	1	1	1	5	1	1	3		1	1		51	44	7	5	
GUARD-HEART	2	3			2	2	2	1	7	1	5		1	1	1	10	2	1		1	4		1	3	1	1	1	1	54	45	9	5	
ITHACA	1	5		1	1	9	3	1	4	2	8		1	2	1	13	1	1	1	1	6	1	3	2	1	1	1	1	72	66	6	2	
MetabERN	4	6	1		1	11	1	1	7	1	10	1	1	1	1	18	1	1	1	1	6	2	3	6	1	2	1	1	91	85	6	1	
PaedCAN	1	4	1	1	2	19	2	2	6	4	7	1	3	2	1	12	1	1	1	1	2	1	6	2	2	3	1	1	90	79	11	0	
RARE-LIVER	1	4			1	10	2	1	3	1	5	1	2	2		13	1	1	2	1	3	1	2	2		2	1		62	52	10	5	
ReCONNET	1	3			1	4	1	1	3	1	7	1	1	1		20	1	1	2	1	6		1	3	1	1	1		63	54	9	5	
RITA	3	2			2	11	2	1	4	1	5	2	1	1	1	14	2	1	1	1	5	1	3	2		2	1	1	70	61	9	3	
TRANSPLANTCHILD	1	2				2	2	1	6	1	2		1	1	1	5	1	1	1	1	3	1	1	3		3			40	33	7	7	
VASCERN	2	5		1		5	3		2	1	4			1	1	6	1	1		1	6	1		1		2	1		45	39	6	9	
Members(Mbrs)	10	94	7	4	46	197	46	5	110	32	148	18	7	6	18	325	17	0	6	0	123	14	44	48	13	45	13	5					
Affiliated Partners(APs)	36	0	1	7	0	0	4	19	1	1	0	0	16	28	0	0	14	24	18	24	0	6	1	0	2	0	11	5					
TOTAL	46	94	8	11	46	197	50	24	111	33	148	18	23	34	18	325	31	24	24	24	123	20	45	48	15	45	24	10				1619	
Areas not covered	0	0	18	13	2	0	0	5	0	0	0	12	7	0	6	0	0	0	4	0	0	7	3	3	13	0	1	15					

2026 ERN Call for Affiliated membres: ONLY in MS with no Member or AP

ERN-EuroBloodNet 4 MS not covered yet: Croatia (HR), Latvia (LV), Norway (NO) and Romania (RO)



ERN's Sustainability beyond 2027

Current Grant: September 2023 - August 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**) is 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 !

Slide courtesy of Ruth Ladenstein, ERNs coordinator's chair, ERN PaedCan, St. Anna Kinderspital Kinderspitalgasse, Vienna



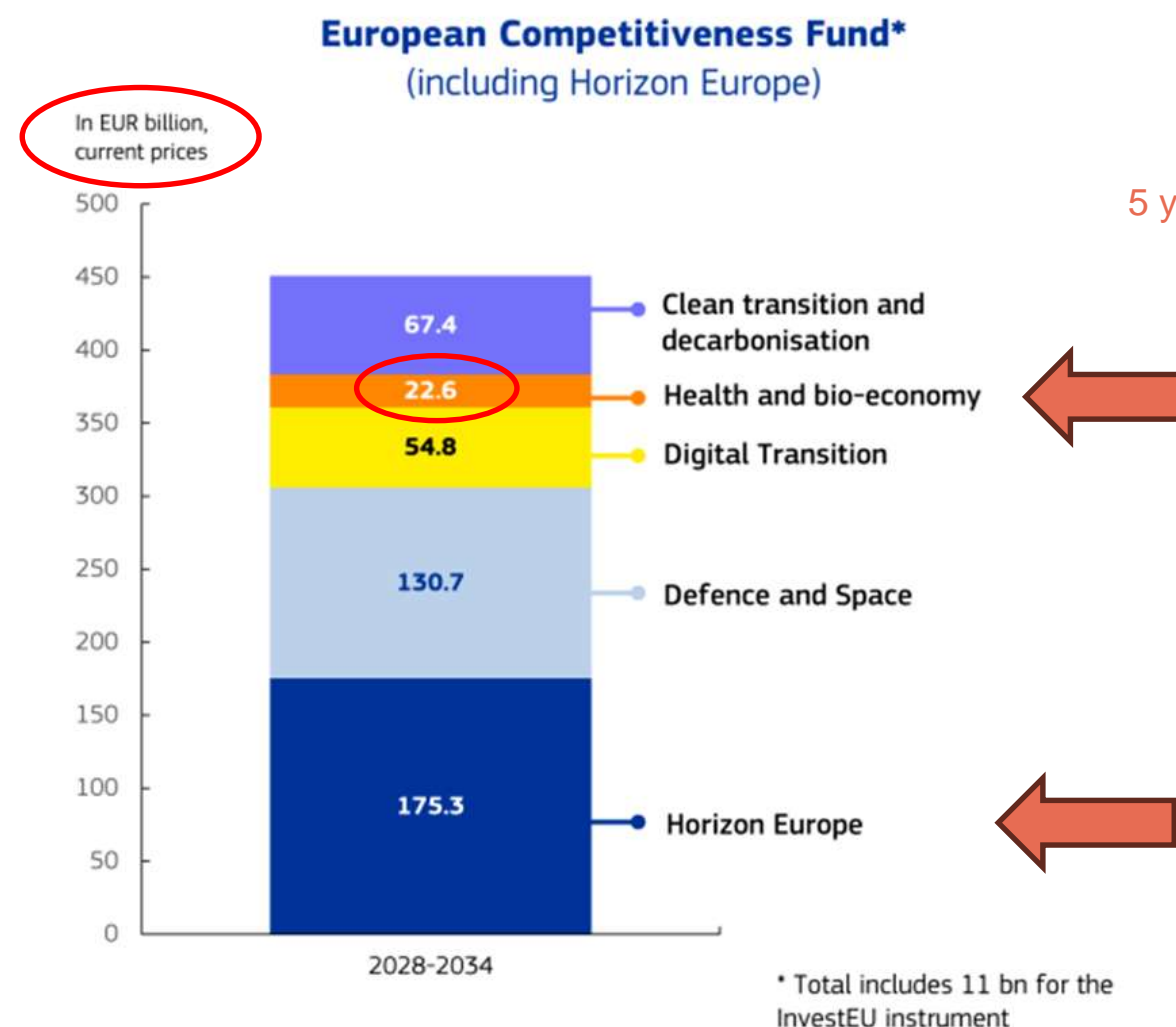
ERN's Sustainability beyond 2027

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**



Remember ERNs Budget in EU4Health:
5 year multi-annual grants €77.4m total, 2023–2027

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

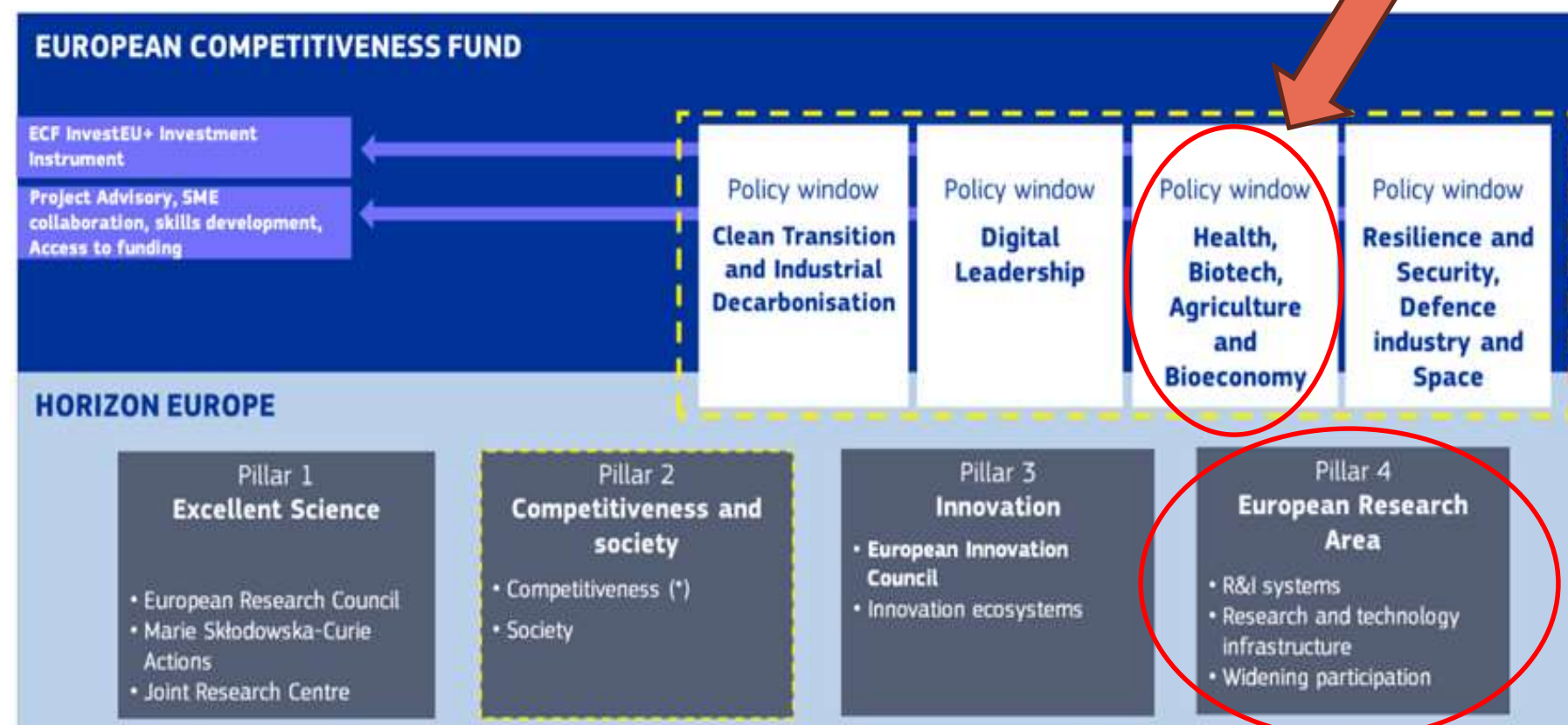
Slide courtesy of Ruth Ladenstein, ERNs coordinator's chair, ERN PaedCan, St. Anna Kinderspital Kinderspitalgasse, Vienna



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.

Slide courtesy of Ruth Ladenstein, ERNs coordinator's chair, ERN PaedCan, St. Anna Kinderspital Kinderspitalgasse, Vienna



ERN's Sustainability beyond 2027

Ongoing strategic communication actions

- ✓ Contributions to information sharing **Webinars organized by DG SANTE**
- ✓ **Success Stories actions** initiated by DG SANTE
- ✓ **Multiannual Financial Framework (MFF)**
 - Letter to the Commissioner for Health European Commission (Mr. Olivér VÁRHELYI).
 - Letters to the national ministries.
 - EC public consultation on MFF - please give your feedback by 12 November 2025.
- ✓ **High Level Meeting** (Brussels; 9-11/12/2025)
- ✓ Participation of ERN Coordinators at the EU Council Conference on Rare Diseases (Cyprus, 5-6 March 2026)
- ✓ Agreement with the Orphanet Journal for Rare Diseases (OJRD) on a Special Issue (Collection of papers related to ERN activities)



ERN's Sustainability beyond 2027



ENGAGE: Rare Diseases and ERNs Must Remain Visible in the EU's Future Budget

Commission adoption

Feedback: Open

Feedback period

18 July 2025 - 12 November 2025 (midnight Brussels time)

The Commission would like to hear your views.

This adopted act is open for feedback for a period of **8 week(s)**. All feedback received will be summarised by the European Commission and presented to the European Parliament and Council with the aim of feeding into the legislative debate. Feedback received will be published on this site and therefore must adhere to the [feedback rules](#).

In order to contribute you'll need to register or login using your existing social media account.

Give feedback >



Sent through email to the HCP Representatives and Substitutes!

We have drafted a:

- **Letter to the President of the EC**
- **Text for the comments section**



ERN's Sustainability beyond 2027



Vytenis Andriukaitis
MEP Lithuania
Host



Maurizio Scarpa
Metab-ERN
B4B Foundation

High-level Agenda for the HLM

	DAY 1 RESEARCH AND INNOVATION	DAY 2 EU INFRASTRUCTURE AND SKILLS	DAY 3 POLICY AND FUNDING
Venue	Residence Palace		European Parliament
Structure of the day	<div>MORNING<ul style="list-style-type: none">High-Level keynote speeches/panelsMaster lecture from ERN Coordinators on the topic of the day</div> <div>AFTERNOON<ul style="list-style-type: none">Panel session 1: success and limitations of the topic of the dayPanel session 2: EU declaration on Rare and Complex diseases</div>		
Topics under discussion (TBC)	<ul style="list-style-type: none">The importance of basic researchConducting clinical trials in the EUTranslational researchResearch on gene therapies and ATMPsPublic-private partnerships for research	<ul style="list-style-type: none">Successes and learnings from the ERNs on joint projectsAdvance Clinical Outcome ResearchEmbedding new diagnostics in care pathwaysThe importance of newborn screeningUse of RWD and AI in clinical applicationBiobanks	<ul style="list-style-type: none">Cross-border healthcareEU Funding mechanisms for R&I in rare diseaseR&I legislative frameworks outside the EUImplications of the WHO Resolution on Rare Diseases
EU Legislations under discussion	EU Pharmaceutical Package EU Regulation on ATMPs EU Clinical Trials Regulation EU Biotech Act	EU Regulation on Health Technology Assessment EU AI Regulation Act European Health Data Space European Union of Skills EU Life Sciences Strategy EU Apply AI Strategy	Multiannual Financial Framework (2028-2034) EU Directive on the application of patients' rights in cross-border healthcare EU Biotech Act EU Critical Medicines Act



ERN's Sustainability beyond 2027

SPEAKERS

DAY 1 - RESEARCH AND INNOVATION

9 December 2025

Residence Palace, Witstraat 155, 1040 Brussels



Vytenis Andriukaitis
Member of the European Parliament (S&D, Lithuania) and host of the High-Level Meeting



Richard Bergstrom
Vice President European Affairs, IQVIA



Michael Berntgen
Head of Scientific Evidence Generation Department, EMA



Niklas Blomberg
Executive Director, Innovative Health Initiative (IHI)



Christophe Clergeau
Member of the European Parliament (S&D, France)



Tanya Collin-Histed
President, Gauchers Association Ltd



Emer Cooke
Executive Director of the European Medicines Agency



Jacques Demotes-Mainard
ECRIN Coordinator



Holm Graessner
European Reference Network for Neurological Disorders (ERN-RND) Coordinator



Manuel Hektor
Former Portuguese Minister of Science, Technology, and Higher Education



Ralf Dieter Hilgers
REALISE-d Coordinator



Virginie Hivert
Acting Chief Executive Officer and Head of Therapies & Access, EURORDIS



Arjan van Hengel
Deputy Head of Unit, Health Innovations & Ecosystems, DG RTD



Daria Jukowska
European Rare Diseases (ERDERA) Coordinator



András Tivadar Kulja
Member of the European Parliament (EPP, Hungary)



Ruth Ladenstein
European Reference Network for Paediatric Oncology (PaedOcan) Coordinator



Mariska Mulder
VP Head of Regulatory Affairs EMEA and LATAM, Ultragenyx



Enrico Letta
President of the Jacques Delors Institute and former President of the Italian Council of Ministers



Alexander Natz
Secretary General of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)



Alberto Pereira
European Reference Network for Rare Endocrine Conditions (Endo-ERN) Coordinator, Adult Chair



Enrico Piccinini
Senior Vice President, EU & International, Rare Diseases, Chiesi Group



Vinciane Pirard
Global Medical Affairs - Rare Diseases, Sanofi



Axel Pries
President, World Health Summit



Maurizio Scarpa
European Reference Network for Metabolic Disorders (MetabERN) Coordinator, President and Founder of Brains for Brain Foundation (Co-Chair)



Enrique Torol
Health Attaché to the Permanent Representation of Spain to the EU



Mark Turner
Chief Executive Officer, ConnectChildren



Sheela Upadhyaya
TogetherRare Steering Group Chair



Aurelijus Veryga
Member of the European Parliament (ECR, Lithuania)

SPEAKERS

DAY 2 - EU INFRASTRUCTURE AND SKILLS

10 December 2025

Residence Palace, Witstraat 155, 1040 Brussels



Dariusz Adamczewski
Managing Director, Children's Tumor Foundation Europe



Vytenis Andriukaitis
Member of the European Parliament (S&D, Lithuania) and host of the High-Level Meeting



Michael Berntgen
Head of Scientific Evidence Generation Department, EMA



Szymon Bielecki
Head of Unit, eHealth, Well-Being and Aging, DG CONNECT



Jim Bonham
President of the International Society of Neonatal Screening



Magda Chlebuz
Executive Director, Science Policy and Regulatory Affairs, EFPIA



Alain Coheur
President of the Single Market, Production and Consumption (INT), European Economic Social Committee



Jane Cooper
Senior Vice-President, Head of EMEA, Ultragenyx



Hélène Dollfus
European Reference Network for rare eye diseases (ERN-EYE) Coordinator



Alessandra Ferlini
ScreeningCare coordinator



Ilaria Galetti
Vice-President, Federation of European Scleroderma Associations & ePAG, ReCONNECT



Liesbet Geris
Professor Biomedical Engineering, University of Leuven, Executive Director VPH Institute



Adam Jarubas
Member of the European Parliament (EPP, Poland)



Ricardo Baptista Leite
CEO, AI Health



Georgios Margetidis
Head of Sector, Health and Food, EU4Health, HUGA



Michael Marmot
Director of the Institute of Health Equity



Martin McKee
Professor of European Public Health at the London School of Hygiene and Tropical Medicine



Marta Mosca
European Reference Network of Connective Tissue and Musculoskeletal diseases (ERN ReCONNECT) Coordinator



Cláran Nicholl
Head of the Health in Society Unit, JCR Research Centre



Michael Ostland
Head of Development Europe, Denali Therapeutics



Nikos Papandreou
Member of the European Parliament (S&D, Greece)



Mar Mañu Pereira
Scientific Coordinator of European Reference Network on Rare Immunodeficiencies (ERIN)



Martine Pergent
President, International Patient Organisation for Primary Immunodeficiencies (IPOP)



Ana Rath
Orphan Director, INSERM



Teresa Ribera
Executive Vice-President for a Clean, Just and Competitive Transition, European Commission



Salla Rinne
Head of Unit on Artificial Intelligence in Health and Life Sciences, DG CONNECT, European Commission



Luca Sangiorgi
European Reference Network on Rare Bone Diseases (ERN BONDI) Coordinator



Maurizio Scarpa
European Reference Network for Metabolic Disorders (MetabERN) Coordinator, President and Founder of Brains for Brain Foundation (Co-Chair)



Tomislav Sokol
Member of European Parliament (EPP, Croatia)



Alberta Spreafico
SDA (Soccorso) - Milan, Digital Innovation Integrated and Translational Care



Anton Ussi
Operations and Finance Director, EATRIS



Vlad Voiculescu
Member of the European Parliament (Renew, Romania)

SPEAKERS

DAY 3 - POLICY AND FUNDING

11 December 2025

European Parliament, Rue de Trèves 1A, 1050 Brussels



Ekaterina Zaharieva
European Commissioner for Startups, Research and Innovation



Olivér Várhegyi
European Commissioner for Health and Animal Welfare



Roberta Metsola
President of the European Parliament



Christel Schaldemose
Member (S&D, Denmark) and Vice-President of the European Parliament



Jennifer Carroll MacNeill
Minister of Health, Ireland



Marija Jakubauskienė
Minister of Health, Lithuania



Vytenis Andriukaitis
Member of the European Parliament (S&D, Lithuania) and host of the High-Level Meeting



Alexis Arzimanoglou
Coordinator of European Reference Network for all rare and complex epilepsies



Stine Bosse
Member of the European Parliament (Renew, Denmark)



Silvio Brusaferro
Former President of the Italian Institute of Health (2012-2023)



Nicolás González Casares
Member of the European Parliament (S&D, Spain) and member of Panel for the Future of Science and Technology (STDA)



Giacomo Chiesi
Head of Global Rare Diseases of the Chiesi Group



Avril Daly
President, EURORDIS



Oxana Doment
WHO Representative to the EU



Joanna Drake
Deputy Director-General for Planet, People and Science for Policy in the European Commission's Directorate-General for Research and Innovation (DG RTD)



Sandra Gallina
Director-General of the Directorate-General for Health and Food Safety (DG SANTE)



Emil Kakkis
CEO Ultragenyx



Peter Liese
Member of the European Parliament (EPP, Germany)



Alexander Natz
Secretary General of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)



Leonidas A. Phylactou
CEO and Medical Director of The Cyprus Institute of Neurology and Genetics



Alexandra Heumber Perry
CEO, Rare Diseases International



Johan Prevot
Executive Director, International Patient Organisation for Primary Immunodeficiencies (IPOP)



Maurizio Scarpa
European Reference Network for Metabolic Disorders (MetabERN) Coordinator, President and Founder of Brains for Brain Foundation (Co-Chair)



Birute Tumiene
Co-Chair of ERN Board of Member States



Till Voigtländer
Coordinator of Joint Action on Integration of ERNs into National Healthcare Systems (IARJIN)



Tiemo Wölken
Member of the European Parliament (S&D, Germany)



ERN's Sustainability beyond 2027

SPEAKERS

DAY 1 - RESEARCH AND INNOVATION
9 December 2025
Residence Palace, Witstraat 155-1040 Brussels

 Vytenis Andriukaitis Member of the European Parliament (S&D, Lithuania) and host of the High-Level Meeting	 Richard Bergstrom Vice President European Affairs, IQVIA	 Mich Bern Head of Evident Depart
 Niklas Blomberg Executive Director, Innovative Health Initiative (IHI)	 Christophe Clergeau Member of the European Parliament (S&D, France)	 Tany Collin Presic Assoc
 Emer Cooke Executive Director of the European Medicines Agency	 Jacques Demotes-Mainard ECRIN Coordinator	 Holm Grae Europe Netw Disord Coord
 Manuel Heitor Former Portuguese Minister of Science, Technology, and Higher Education	 Ralf Dieter Hilgers REALISE-d Coordinator	 Virgii Hiver Acting Officer, Therap, EURCA
 Arjan van Hengel Deputy Head of Unit, Health Innovations & Ecosystems, DG RTD	 Daria Julkowska European Rare Diseases Research Alliance (ERDRA) Coordinator	 Andr Kulja Member, Parli Hunga
 Ruth Ladenstein European Reference Network for Paediatric Oncology (PaedOnc) Coordinator	 Mariska Mulder VP Head of Regulatory Affairs EMEA and LATAM, Ultragenyx	 Enrico Letta Preside Jacque Instituti Preside Council
 Alexander Natz Secretary General of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)	 Alberto Pereira European Reference Network for Rare Endocrine Conditions (Endo-ERN) Coordinator, Adult Chair	 Enric Picci Senior EU & I Diseases
 Vinciane Pirard Global Medical Affairs - Rare Diseases, Sanofi	 Axel Pries President, World Health Summit	 Maur Scarj Europe Netw Disord Coord Found
 Enrique Torol Health Attaché to the Permanent Representation of Spain to the EU	 Mark Turner Chief Executive Officer, ConnectChildren	 Shee Upat Togit Group
 Aurelijus Veryga Member of the European Parliament (ECR, Lithuania)		

High-Level Meeting on Rare Diseases 2025

9-11 December, Brussels

[Register now](#)

Join us for a high-level, three-day meeting dedicated to forging a robust research & innovation ecosystem for rare diseases in the EU. This event will gather key stakeholders — including EU policymakers, industry leaders, patient advocacy groups, researchers, and healthcare providers — to identify concrete actions and incentives to enhance innovation across the rare disease ecosystem.

By involving all actors in the development, approval, and access to new therapies, we aim to establish a predictable regulatory pathway, ensuring that the benefits of scientific advancements reach all European citizens, particularly those often left behind.

A central goal of the HLM Rare will be advocating for the EU's adoption of a **European Declaration on Rare and Complex Diseases**, aiming to establish a sustainable Research and Innovation Ecosystem that ensures continuous dialogue and collaboration among all stakeholders.

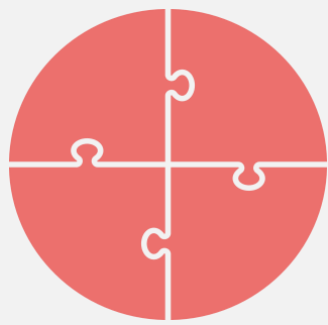
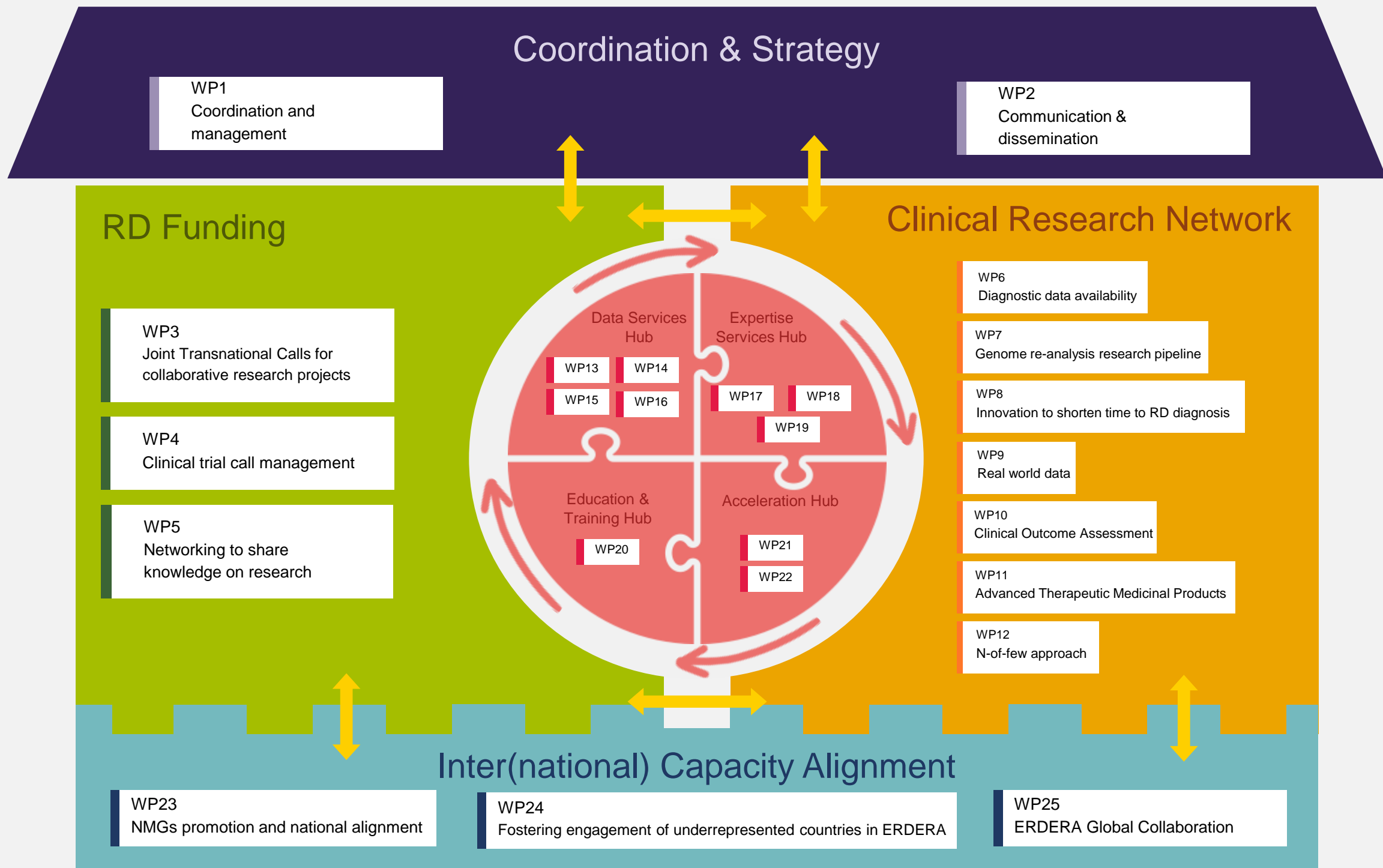
[Discover more](#)

PEAKERS

POLICY AND FUNDING
10-11 December 2025
Parliament, Rue de Trèves 11A, 1050 Brussels

 Ekaterina Zaharieva European Commissioner for Startups, Research and Innovation	 Olivér Várhegyi European Commissioner for Health and Animal Welfare	 Roberta Metsola President of the European Parliament
 Christel Schaldemose Member (S&D, Denmark) and Vice-President of the European Parliament	 Jennifer Carroll MacNeill Minister of Health, Ireland	 Marija Jakubauskienė Minister of Health, Lithuania
 Vytenis Andriukaitis Member of the European Parliament (S&D, Lithuania) and host of the High-Level Meeting	 Alexis Arzimanoglou Coordinator of European Reference Network for all rare and complex epilepsies	 Stine Bosse Member of the European Parliament (Renew, Denmark)
 Silvio Brusaferro Former President of the Italian Institute of Health (2019-2023)	 Nicolás González Casares Member of the European Parliament (S&D, Spain) and member of Panel for the Future of Science and Technology (STOA)	 Giacomo Chiesi Head of Global Rare Diseases of the Chiesi Group
 Avril Daly President, EURORDIS	 Oxana Domentii WHO Representative to the EU	 Joanna Drake Deputy Director-General for Planet, People and Science for Policy in the European Commission's Directorate-General for Research and Innovation (DG RTD)
 Sandra Gallina Director-General of the Directorate-General for Health and Food Safety (DG SANTE)	 Emil Kakkis CEO Ultragenyx	 Peter Liese Member of the European Parliament (EPP, Germany)
 Alexander Natz Secretary General of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)	 Leonidas A. Phylactou CEO and Medical Director of The Cyprus Institute of Neurology and Genetics	 Alexandra Heumber Perry CEO, Rare Diseases International
 Johan Prevot Executive Director, International Patient Organisation for Primary Immunodeficiencies (IPOPI)	 Maurizio Scarpa European Reference Network for Metabolic Disorders (MetabERN) Coordinator, President and Founder of Brains for Brain Foundation (Co-Chair)	 Birute Tumiene Co-Chair of ERN Board of Member States
 Till Voigtländer Coordinator of Joint Action on Integration of ERNs into National Healthcare Systems (IARINE)	 Timo Wölken Member of the European Parliament (S&D, Germany)	





- 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

CLINICAL RESEARCH NETWORK _ Outcome research

Real World Data

- **Task 9.1** – Use of primary healthcare data (EHRs) for RD outcome research
- **Task 9.2** – Use of population-based data for RD outcome research
- **Task 9.3** – Integrating patient cohorts for natural history / standard-of-care reference studies
- **Task 9.4** – Development of a blueprint and inventory of regulatory-grade natural history cohort data
- **Task 9.5** – Disease progression modelling and prognostic biomarker research
- **Task 9.6** – Development of a regulatory grade clinical trial simulation platform for rare diseases

Clinical Outcome Assessment

- **Task 10.1** – Platform for regulatory-grade patient-centred COA development and validation
- **Task 10.2** – Development and Implementation of Clinical Outcome Assessment Tools
- **Task 10.3** – Unveiling the Hidden Burden: Estimating the Socioeconomic Impact of Rare Diseases for Informed Decision Making and Resource Allocation

T9.1: eUROGEN; EURO-NMD; CRANIO, EpiCare, EuroBloodNet;
T9.2: EpiCare; ERNICA; MetabERN; T9.3: ITHACA, ERKNet, ERNs ENDO-BOND;
T9.4: RND, EuroBloodNet, ERKNet, EYE; T9.5: EURO-NMD; T9.6: ERKnet; DDF

T10.1: RND; ITHACA; mito-InterERN workgroup (EURO-NMD, RND, MetabERN, Eye, EpiCare); EuroBloodNet; CRANIO;
T10.2: EpiCare; EURO-NMD; ERKNet; EuroBloodNet; ERN-RND



NAME	SUBGROUPS	GOVERNANCE STRUCTURE		BoMS / ERNs MEMBERSHIP	MANDATE
Ethical Legal and Social Implications (ELSI)	N/A	Chair	Luca Sangiorgi (ERN BOND)	<ul style="list-style-type: none"> – BoMS Co-Chair – BoMS AT – BoMS BE – BoMS CZ – BoMS EL – BoMS ES – BoMS FR – BoMS LT – BoMS MT – BoMS NL – BoMS PL – BoMS RO – Endo-ERN – ERN BOND – ERN EpiCARE – ERN EURACAN – ERN eUROGEN – ERN GENTURIS – ERN PaedCan – ERN RARE-LIVER – ERN-EYE – ERNICA 	– The WG's mandate has been finalised
		Co-Chairs	Nichola Larkins (EC) Natalia Zampieri (EC)		



ERN's WGs

NAME	SUBGROUPS	GOVERNANCE STRUCTURE		BoMS / ERNs MEMBERSHIP	MANDATE
				– VASCERN	
Management Activities (MA) ¹	1. Registries <ul style="list-style-type: none"> – Endo-ERN – ERKNet – ERN BOND – ERN CRANIO – ERN EpiCARE – ERN EURACAN – ERN eUROGEN – ERN EURO-NMD – ERN GENTURIS – ERN GUARD-Heart – ERN RARE-LIVER – ERN ReCONNET – ERN RITA – ERN TRANSPLANT-CHILD – ERN-EuroBloodNet – ERN-EYE – ERN-ITHACA – ERN-LUNG – ERN-RND – ERN-Skin – ERNICA – MetabERN – VASCERN 	Chair	Michelle Battye (ERN eUROGEN) Diana Marinello (ERN ReCONNET) Charlotte Van Beuzekom (Endo-ERN)	– Endo-ERN – ERKNet – ERN BOND – ERN CRANIO – ERN EpiCARE – ERN EURACAN – ERN eUROGEN – ERN EURO-NMD – ERN GENTURIS – ERN GUARD-Heart – ERN PaedCan – ERN RARE-LIVER – ERN ReCONNET – ERN RITA – ERN TRANSPLANT-CHILD – ERN-EuroBloodNet – ERN-EYE – ERN-ITHACA – ERN-LUNG – ERN-RND – ERN-Skin – ERNICA – MetabERN – VASCERN	– The WG's mandate has been finalised
	2. Communication activities <ul style="list-style-type: none"> – Endo-ERN – ERKNet – ERN BOND 	Co-Chairs			
	3. CPMS (Clinical Patient Management System)				



NAME	SUBGROUPS	GOVERNANCE STRUCTURE		BoMS / ERNs MEMBERSHIP	MANDATE
Improvement of the Evaluation (EI)	N/A	Chair	Hana Horka (EC)	<ul style="list-style-type: none"> – BoMS Co-Chair – BoMS BE – BoMS CZ – BoMS FR – BoMS HU – BoMS LV – BoMS NO – BoMS PL 	– The WG's mandate has been finalised
		Co-Chairs	Alexis Arzimanoglou (ERN EpiCARE) Pavla Doležalová (CZ BoMS)		
Monitoring (MO)	N/A	Chair	Franz Schaefer (ERKNet)	<ul style="list-style-type: none"> – Endo-ERN – ERKNet – ERN CRANIO – ERN EpiCARE – ERN EURACAN – ERN eUROGEN – ERN EURO-NMD – ERN PaedCan – ERN RARE-LIVER – ERN ReCONNET – ERN RITA – ERN TRANSPLANT-CHILD – ERN-EuroBloodNet – ERN-ITHACA – ERN-LUNG – ERN-RND – ERNICA – VASCERN 	
		Co-Chairs	Alexis Arzimanoglou (ERN EpiCARE) Joao de Sousa (EC)		
Transition (TR)	N/A	Chairs	To be nominated	<ul style="list-style-type: none"> – ERN Youth Panel representative – Endo-ERN – ERKNet – ERN BOND – ERN CRANIO – ERN EpiCARE – ERN EURACAN – ERN EURO-NMD 	– The WG's DRAFT mandate has been finalised



ERN's WGs

NAME	SUB GROUPS	GOVERNANCE STRUCTURE		BoMS / ERNs MEMBERSHIP	MANDATE
Knowledge Generation and capacity (KG)	1. Erasmus+ Programme & ERNs <ul style="list-style-type: none"> – Endo-ERN – ERKNet – ERN BOND – ERN EpiCARE – ERN eUROGEN – ERN RARE-LIVER – ERN-ITHACA – ERN-Skin – MetabERN – VASCERN 	Chair	Maurizio Scarpa (MetabERN)	BoMS ES BoMS HU BoMS IE BoMS PT Endo-ERN ERKNet ERN BOND ERN CRANIO ERN EpiCARE ERN EURACAN ERN eUROGEN ERN EURO-NMD ERN GENTURIS ERN PaedCan ERN RARE-LIVER ERN ReCONNET ERN RITA ERN TRANSPLANT-CHILD ERN-EuroBloodNet ERN-EYE ERN-ITHACA ERN-LUNG ERN-RND ERN-Skin ERNICA MetabERN VASCERN	– The WG's mandate has been finalised
	2. Academic/specialisation courses on rare diseases <ul style="list-style-type: none"> – ERN EpiCARE – ERN RARE-LIVER – ERN-ITHACA – ERN-LUNG – MetabERN 				
	3. ERN Academy <ul style="list-style-type: none"> – Endo-ERN – ERKNet – ERN CRANIO – ERN EpiCARE – ERN eUROGEN – ERN RITA – ERN-EYE – ERN-ITHACA – ERN-LUNG – MetabERN – VASCERN 				
	4. Artificial Intelligence & rare diseases <ul style="list-style-type: none"> – Endo-ERN 	Co-Chairs	György Pfliegler (HU BoMS) Stefano Vettorazzi (EC)		

Thank you!

