European Reference Networks

Group of the Progressive Alliance of Socialists & Democrats in the European Parliament

BRAINS FOR BRAIN

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HLM RARE 2025

Building a European Innovation and Care Ecosystem for Rare and Complex Diseases

9-11 December, Brussels

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WELCOME MESSAGE FROM THE HOSTS

Welcome message from Prof. Maurizio Scarpa

Coordinator of European Reference Network for Hereditary Metabolic Diseases (MetabERN), and Founder of Brains for Brain Foundation (Co-Chair)

It is my great pleasure to welcome you to the High-Level Meeting on Rare Diseases 2025, co-organised by the Brains for Brain Foundation, MEP Vytenis Andriukaitis (S&D, Lithuania) and the European Reference Networks (ERNs) for rare and complex diseases. This gathering brings together an exceptional community of policy-makers, researchers, clinicians, industry leaders and patient representatives, united by a shared commitment to transforming the landscape for people living with rare and complex diseases in Europe.

Rare diseases remain a powerful example of unmet medical need. Across Europe, more than 30 million people live with a rare and complex disease. Yet 95% of these conditions currently have no approved treatment, and patients often face years-long diagnostic delays, fragmented care pathways and unequal access to innovation. While the past 15 years have seen major EU investments and programmes, these efforts remain too often disconnected. It is now time to align and strengthen them to build a truly integrated ecosystem where collaboration accelerates innovation and delivers real benefits to patients and families.

Guided by the Draghi, Letta and Heitor reports, this High-Level Meeting will culminate with a Declaration on the EU Innovation and Care Ecosystem for Rare and Complex Diseases, a bold commitment to a sustainable research and innovation ecosystem supported by dedicated funding. Furthermore, over the next three days, our agenda will focus on unleashing research and innovation, mobilising strategic investments and strengthening Europe's global competitiveness in the rare disease field.

I warmly thank you for your participation and engagement. Together, we can turn collective ambition into concrete action for the millions of people living with rare and complex diseases across Europe!

Welcome message from Vytenis Andriukaitis

Member of the European Parliament (Progressive Alliance of Socialists and Democrats, Lithuania)

A Call to Action: Igniting the European Ecosystem for Rare and Complex Diseases

The situation in the European Union regarding the problem of rare diseases is now doomed. On the one hand, we have systems in place such as the European Reference Networks (ERNs), the European Alliance for Health Data Centres (EAHDCs), Artificial Intelligence (AI) Factories, biobanks and bio registries, and the 1 million Genomics Project. However, they are all operating separately. On the other hand, we have a problem with trials: in the UK, a clinical trial runs for 60 days; in the US, 30 days; and in Europe, 82 days on average. Finally, 30–36 million people across Europe are living with rare diseases. These are not just numbers; they represent the personal stories of families and individuals searching for answers and help. As you can see, the situation is complicated; therefore, we must step up and act immediately.

The alarming consensus from the Draghi, Heitor, Niinistö and Letta reports is clear: Europe is now lagging, held back by fragmentation that causes us to lose vital clinical trials, investment, and—most critically—new therapies for patients. While the General Pharmaceutical Legislation Review is essential, it is not a panacea for the fundamental challenge of delivering research breakthroughs and therapies across 27 Member States.

We cannot wait for a single piece of regulation to solve a systemic problem. We must complement legislation with coordinated, high-ambition action.

This is our call to establish a European Ecosystem for Research and Innovation for rare and complex diseases.

To overcome the challenges we are facing, we need to align existing programmes and frameworks, integrate health innovation across all sectors and bring research and funding together. Only then can we unlock brand new therapies and treatment and detection technologies. Our strength lies in the diversity and quality of our health data.

This bespoke, Critical Medicines Alliance-style framework must move immediately to strengthen the ERNs, ensure their interoperability, and fully realize their promise in translating research into crossborder care. It demands an even stronger European Medicines Agency, empowered with a dedicated focus on the unique challenges of rare diseases, and it requires

the courage to invest and share risks. That is why it is so crucial that there is a more joint-up approach between Commission Directorates and relevant regulatory agencies.

The next Multiannual Financial Framework offers a unique opportunity. If the EU is to make an impact and reverse the worrying trends of reduced clinical trials and availability of Advanced Therapeutic Medicinal Products, we must ensure the MFF includes a horizontal flagship initiative on rare and complex diseases within Horizon and the Competitiveness Innovation Fund. This initiative must move beyond isolated research approaches to fund ambitious, long-term projects that are willing to share and learn from failures, scale-up successes, ultimately enabling the full translation from research to patient access and building our pan-European infrastructure.

Rare and complex diseases have always been the lynchpin for the European Health Union. To succeed, we cannot afford to be bound by obsolete frameworks. We must embrace the courage to take risks, innovate differently, and leverage the deep expertise that already exists across the EU. This requires building more flexible frameworks that reward bravery and allow us to advance rapidly alongside stakeholders open to novel solutions—be it through pre-clinical open source data sharing, joint procurement, or exploring unified price-setting models.

The High-Level Meeting RARE is the essential platform to kickstart these discussions, prioritize the most impactful actions, and define the supportive framework that will enable this research and innovation ecosystem. By acting boldly, we will gain trust, deliver tangible results for our citizens, and truly advance the European Health Union. The time for delay is over. The time to align, act and accelerate is now.

Welcome message from Prof. Ruth Ladenstein

Coordinator of European Reference Network for Paediatric Oncology (PaedCan); and TRIO Chair, European Reference Networks (ERNs)

As the TRIO Chair of the European Reference Networks (ERNs), I would like to welcome you to the HLM Rare 2025, an important milestone for the entire rare disease community. This important meeting offers us a unique opportunity to reflect on our achievements, to strengthen our collaborations, and to define the next steps in building a more equitable future for all patients living with rare and complex diseases across Europe.

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The ERNs are a cornerstone of the rare disease ecosystem. By uniting specialised expertise across Europe and connecting more than 1,600 expert centres, they help reduce geographical inequalities, improve diagnosis and care, and support innovative, cross-border research.

This is why I believe we are ready for the next important step in the evolution of the ERNs: strengthening national structures that can bring together the essential elements of research, innovation and care for rare diseases. This would build naturally on the progress we have made and support a more coordinated and accessible approach for rare disease communities.

Recognising the pivotal role of the ERNs in addressing the substantial burden of undiagnosed rare diseases is equally important. Their concentrated expertise and cross-border collaboration help shorten diagnostic journeys and bring clarity and hope to those still seeking answers.

The ERNs must also be empowered to further connect the rare disease ecosystem across the full research, diagnostic and care spectrum, while continuing to serve as a vital educational hub for the next generation of specialists and researchers. The educational and knowledge-sharing capacities embedded in our networks remain among our greatest assets.

At the same time, we must properly recognise and support the researchers, medical teams and patient advocates who drive progress in this field. Valuing their contributions is essential to advancing innovation and improving the lives of patients.

We must also continue to support the essential voice of patient advocacy. Adequate resources enable patient organisations to contribute fully to their many tasks and advisory roles at national and European levels.

The HLM Rare 2025 offers a crucial opportunity to address these important issues together and to shape the future direction of rare disease policy and collaboration in Europe. Therefore, I would like to thank all participants for your engagement, your expertise and your unwavering dedication. I wish you an inspiring and productive meeting.

Co-funded by the European Union
Grant Agreement - GAP-101233680

THE HIGH-LEVEL MEETING ON RARE DISEASES

In the EU, an estimated 27 to 36 million people live with a rare disease. For many, getting a diagnosis takes years; for others, daily disease management becomes a heavy burden. Access to effective therapies remains the greatest challenge, with more than 95% of rare disease patients currently lacking any available treatment. This stark reality highlights the urgent need for a stronger, more coordinated European response.

The High-Level Meeting (HLM) on Rare Diseases seeks to meet this challenge. Bringing together EU policymakers, industry leaders, the 24 European Reference Networks, patient organisations, researchers, and healthcare providers, the HLM will serve as a platform to identify concrete actions and incentives to foster innovation across the entire rare disease ecosystem.

At the heart of the meeting lies the ambition to secure EU adoption of a European Declaration on Rare and Complex Diseases, a political commitment to establish a sustainable research and innovation ecosystem and to create a new consultative mechanism that guarantees continuous dialogue among stakeholders.

Inspired by the Draghi, Letta, and Heitor reports, and guided by the expertise of the European Reference Networks (ERNs), the HLM will explore ways to reduce fragmentation, attract long-term investment, and accelerate the pathway from discovery to patient access. By addressing both financial and non-financial incentives, the meeting aims to strengthen Europe's position as a leader in rare disease research and innovation, ensuring that scientific progress translates into real impact for millions of patients and families.

OUR ENDORSERS & SPONSORS

The organisation of the High-Level Meeting and the development of the European Declaration on Rare and Complex Diseases have been shaped through a rigorous, multi-stakeholder process designed to reflect the realities, challenges, and opportunities of the rare disease community across Europe. Central to this process is the Steering Committee, composed of distinguished leaders from ERNs, patient organisations, academia and industry. This diverse group of sponsors and endorsers provided strategic direction for the HLM and oversaw the drafting of the Declaration, ensuring that it captures the shared ambitions of the European rare disease community.

We are deeply grateful to the many organisations and partners whose commitment made this event possible. Their support enabled us to bring together the European and global rare disease community to share knowledge, foster innovation and work towards improving the lives of the millions of people living with rare and complex diseases.

FUNDING

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This project has received funding from the European Union, through the EU4Health Programme (EU4H) call EU4H-2024-PJ-03).

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EUROPEAN REFERENCE NETWORK ON RARE BONE DISEASES

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EVENT AGENDA

DAY 1: RESEARCH & INNOVATION

Welcome speeches	
09:20 – 09:35	**Maurizio Scarpa** Director Regional Coordinating Centre for Rare Diseases, University Hospital Udine, Coordinator European Reference Network for Hereditary Metabolic Diseases (MetabERN) and Founder of Brains for Brain Foundation (Co-Chair)
09:35 – 09:50	**Vytenis Andriukaitis** Member of the European Parliament (S&D, Lithuania) and host of the High-Level Meeting
Keynote Speeches	
09:50 – 10:00	**Hans Kluge** Regional Director for Europe, World Health Organization
10:00 – 10:15	**Enrico Letta** President of the Jacques Delors Institute and former President of the Italian Council of Ministers
10:15 – 10:30	**Manuel Heitor** Former Portuguese Minister of Science, Technology, and Higher Education
10:30 – 10:45	**Emer Cooke** Executive Director of the European Medicines Agency
10:45 – 11:00	**Marina Zanchi** Director of the European Health and Digital Executive Agency (HaDEA)
11:00 - 11:15	**Richard Bergstrom** Vice President European Affairs, IQVIA
11:15– 11:45	**Coffee break**
Panel Discussions	
11:45 – 12:45	**PANEL DISCUSSION I** **The Future of Rare Disease Innovation: Can Europe Overcome Fragmentation and Regain Global Leadership?** Moderated by Josep Figueras, Director Emeritus of European Observatory on Health Systems and Policies ▪ **Emer Cooke** Executive Director of the European Medicines Agency ▪ **Manuel Heitor** Former Portuguese Minister of Science, Technology, and Higher Education

	▪ **Mariska Mulder** VP Head of Regulatory Affairs EMEA and LATAM, Labelling & Marketed Products, Ultragenyx ▪ **Axel Pries** President, World Health Summit ▪ **Maurizio Scarpa** Director Regional Coordinating Centre for Rare Diseases, University Hospital Udine, Coordinator European Reference Network for Hereditary Metabolic Diseases (MetabERN) and Founder of Brains for Brain Foundation (Co-Chair) ▪ **Aurelijus Veryga** Member of the European Parliament (ECR, Lithuania)
12:45-14:00	**PANEL DISCUSSION II** **Fostering EU leadership in clinical trials for rare diseases** Moderated by Josep Figueras, Director Emeritus of European Observatory on Health Systems and Policies ▪ **Michael Berntgen** Head of Scientific Evidence Generation Department, EMA ▪ **Niklas Blomberg** Executive Director, Innovative Health Initiative (IHI) ▪ **Holm Graessner** European Reference Network for Neurological Disorders (ERN-RND) Coordinator ▪ **Ralf Dieter Hilgers** RealiseD Coordinator ▪ **Virginie Hivert** Acting Chief Executive Officer and Head of Therapies & Access, EURORDIS-Rare Diseases Europe ▪ **Daria Julkowska** European Rare Diseases Research Alliance (ERDERA) Coordinator ▪ **Enrico Piccinini** Senior Vice President, EU & International, Rare Diseases, Chiesi Group
14:00 – 15:00	**Lunch break**
15:00 - 15:10	**Adam Jarubas** Member of the European Parliament (EPP, Poland)
15:10-16:25	**PANEL DISCUSSION III** **Creating comprehensive rare disease infrastructure clusters (CoRDIC) for research, innovation and care** Moderated by Ruth Ladenstein, European Reference Network for Paediatric Oncology (PaedCan) Coordinator ▪ **Jacques Demotes-Mainard** ECRIN Director General

	▪ **Arjon van Hengel** Senior Policy Officer, Health Innovations & Ecosystems, DG RTD ▪ **Holm Graessner** European Reference Network for Neurological Disorders (ERN-RND) Coordinator ▪ **Virginie Hivert** Acting Chief Executive Officer and Head of Therapies & Access, EURORDIS-Rare Diseases Europe ▪ **Georgios Margetidis** Head of Sector, EU4Health grants on cancer, NCDs, mental health, rare diseases and health workforce, HaDEA ▪ **Alexander Natz** Secretary General, EUCOPE ▪ **Mark Turner** Chief Executive Officer, Conect4Children Stichting ▪ **Sheela Upadhyaya** Together4Rare Steering Group Chair
16:25 – 16:55	**Coffee break**
16:55 - 18:00	**PANEL DISCUSSION IV** **Ringfencing EU funding for ERNs and translational research** Moderated by Holm Graessner, European Reference Network for Neurological Disorders (ERN-RND) Coordinator ▪ **Sonata Jarmalaite** Adviser to the President of the Republic of Lithuania ▪ **Ruth Ladenstein** European Reference Network for Paediatric Oncology (PaedCan) Coordinator ▪ **Alberto Pereira** European Reference Network for Rare Endocrine Conditions (Endo – ERN) Coordinator, Adult Chair ▪ **Vinciane Pirard** Global Medical Affairs – Rare Diseases, Sanofi ▪ **Enrique Terol** Health Attaché to the Permanent Representation of Spain to the EU
18:00 - 18:30	**Final considerations** ▪ **Vytenis Andriukaitis,** Member of the European Parliament (S&D, Lithuania) and host of the High-Level Meeting ▪ **Holm Graessner**, European Reference Network for Neurological Disorders (ERN-RND) Coordinator ▪ **Ruth Ladenstein**, European Reference Network for Paediatric Oncology (PaedCan) Coordinator

DAY 2: EU INFRASTRUCTURE & SKILLS

Welcome Speeches

09:00 – 09:05	**Maurizio Scarpa** Director Regional Coordinating Centre for Rare Diseases, University Hospital Udine, Coordinator European Reference Network for Hereditary Metabolic Diseases (MetabERN) and Founder of Brains for Brain Foundation (Co-Chair)
09:05 – 09:10	**Vytenis Andriukaitis** Member of the European Parliament (S&D, Lithuania) and host of the High-Level Meeting

Master lecture

09:10 – 09:25	**Michael Marmot** Director of the Institute of Health Equity

Keynote Speeches

09:25 – 09:35	**European Health Data Space** **Tomislav Sokol** Member of the European Parliament (EPP, Croatia)
09:35 – 09:45	**Application of AI in healthcare in the EU** **Saila Rinne** Head of Unit on Artificial Intelligence in Health and Life Sciences, DG CONNECT, European Commission
09:45 - 09:55	**National and Pan-EU Infrastructures for Rare and Complex Diseases** **Konstantinos Kleovoulou** Director of Research and Innovation, Deputy Ministry of Research, Innovation and Digital Policy, Cyprus

Panel Discussions

09:55 – 11:00

PANEL DISCUSSION I

Resilience and Reliability: Securing Rare Disease Diagnosis and Care in a Changing World

Moderated by Tamsin Rose, Professional Moderator

- **Alain Coheur**
  President Commission on Industrial Change, European Economic and Social Committee
- **Hélène Dollfus**
  European Reference Network for rare eye diseases (ERN-EYE) Coordinator
- **Ilaria Galetti**
  Vice-President Federation of European Scleroderma Associations & ePAG ReCONNET
- **Liesbet Geris**

Professor Biomedical Engineering, Université of Liège and KU Leuven; Executive Director VPH Institute, Belgium

- **Jens K. Habermann**
  Director General BBMRI-ERIC
- **Saila Rinne**
  Head of Unit on Artificial Intelligence in Health and Life Sciences, DG CONNECT, European Commission

11:00 – 11:30 **Coffee break**

PANEL DISCUSSION II
Strengthening Workforce Development and Capacity Building for rare diseases and formalizing the ERN academy
Moderated by Tamsin Rose, Professional Moderator

11:30 – 12:45

- **Christophe Clergeau**
  Member of the European Parliament (S&D, France)
- **Ilaria Galetti**
  Vice-President Federation of European Scleroderma Associations & ePAG ReCONNET
- **Jens K. Habermann**
  Director General BBMRI-ERIC
- **Donata Meroni**
  Head of Unit B3 'Health monitoring and cooperation, Health networks', European Commission
- **Marta Mosca**
  European Reference Network of Connective Tissue and Musculoskeletal diseases (ERN ReCONNET) Coordinator
- **Maurizio Scarpa**
  Director Regional Coordinating Centre for Rare Diseases, University Hospital Udine, Coordinator European Reference Network for Hereditary Metabolic Diseases (MetabERN) and Founder of Brains for Brain Foundation (Co-Chair)
- **Alberta M.C. Spreafico**
  Senior Vice President Health Innovation, EVERSANA; President, Innovation for Global Health Institute

12:45 – 13.00

Fireside chat 'Connecting stakeholders for impact: building the infrastructure of a modern rare disease Europe'

- **Vytenis Andriukaitis**
  Member of the European Parliament (S&D, Lithuania) and host of the High-Level Meeting
- **Nathalie Moll**
  Director General of the European Federation of Pharmaceutical Industries and Associations
- **Maurizio Scarpa**
  Director Regional Coordinating Centre for Rare Diseases, University Hospital Udine,

	Coordinator European Reference Network for Hereditary Metabolic Diseases (MetabERN) and Founder of Brains for Brain Foundation (Co-Chair)
13:00 – 14:00	**Lunch**
Keynote Speech	
14:00 – 14.10	**Martin McKee** Professor of European Public Health at the London School of Hygiene and Tropical Medicine (video message)
14:10– 14:25	**Teresa Ribera** Executive Vice-President for a Clean, Just and Competitive Transition, European Commission
14:25 – 14:35	**Ricardo Baptista Leite** CEO, HealthAI (video message)
Panel Discussions	
14:35 – 15:55	**PANEL DISCUSSION III** **Accelerating equitable access to screening programmes, advanced diagnostics and innovative orphan drugs** Moderated by Luca Sangiorgi, European Reference Network on Rare Bone Diseases (ERN-BOND) Coordinator ▪ **James R. Bonham** President of the International Society of Neonatal Screening (ISNS) ▪ **Jane Cooper** Senior Vice-President, Head of EMEA, Ultragenyx ▪ **Alessandra Ferlini** University of Ferrara, Italy & Scientific Coordinator of the EU-IHI Screen4Care project ▪ **Peter Mulders** European Reference Network for Rare Uro-Recto-Genital Diseases and Complex Conditions (EUROGEN) Coordinator ▪ **Martine Pergent** President, International Patient Organisation for Primary Immunodeficiencies (IPOPI) ▪ **Anton Ussi** Operations and Finance Director, EATRIS
15:55 – 16:25	**Coffee break**
16:25 – 16.30	**Kim Angel** Executive Director at International MPS Network (video message)
16:30 – 16.50	**FIRESIDE CHAT** • **Mar Mañu Pereira**

European Reference Network on Rare Hematological Diseases (ERN-EuroBloodNet) Coordinator

- **Ciarán Nicholl**
  Head of the Health in Society Unit, Joint Research Centre

PANEL DISCUSSION IV

Boosting high-quality, interoperable rare-disease data and real-world evidence under the EHDS

Moderated by Mar Mañu Pereira, European Reference Network on Rare Hematological Diseases (ERN-EuroBloodNet) Coordinator

16:50 – 18:05

- **Christel Schaldemose**
  Vice-President, European Parliament
- **Dariusz Adamczewski**
  Managing Director, Children's Tumor Foundation Europe
- **Michael Berntgen**
  Head of Scientific Evidence Generation Department, EMA
- **Szymon Bielecki**
  Head of Sector, AI health tech and infrastructure, DG CONNECT
- **Teresinha Evangelista**
  European Reference Network for neuromuscular diseases (ERN EURO-NMD) Coordinator
- **Michael Ostland**
  Head of Development Europe, Denali Therapeutics
- **Ana Rath**
  Orphanet Director, INSERM

Final considerations

Presented by

18:05– 18:30

- **Mar Mañu Pereira**
  European Reference Network on Rare Hematological Diseases (ERN-EuroBloodNet) Coordinator
- **Luca Sangiorgi**
  European Reference Network on Rare Bone Diseases (ERN-BOND) Coordinator,
- **Maurizio Scarpa**
  Director Regional Coordinating Centre for Rare Diseases, University Hospital Udine, Coordinator European Reference Network for Hereditary Metabolic Diseases (MetabERN) and Founder of Brains for Brain Foundation (Co-Chair)

DAY 3: POLICY & FUNDING

Welcome Speeches

09:00 – 09:15	**Vytenis Andriukaitis** Member of the European Parliament (S&D, Lithuania)
09:15 – 09:30	**Emil Kakkis** CEO Ultragenyx (online attendance)

Keynote Speeches:
Breaking Down Barriers: Accelerating Rare Disease Therapies Through a Coherent Regulatory Framework

09:30 – 09:35	**Roberta Metsola** President of the European Parliament (video message)
09:35 – 09:45	**Tiemo Wölken** Member of the European Parliament (S&D, Germany)
09:45 – 10:00	**Maurizio Scarpa** Director Regional Coordinating Centre for Rare Diseases, University Hospital Udine, Coordinator European Reference Network for Hereditary Metabolic Diseases (MetabERN) and Founder of Brains for Brain Foundation (Co-Chair)
10:00 – 10:15	**Ekaterina Zaharieva** European Commissioner for Startups, Research and Innovation
10:15 – 10:30	**Industry Perspective on a competitive, innovative and equitable EU ecosystem for rare and complex diseases** • **Giacomo Chiesi** Executive Vice President, Global Rare Diseases, Chiesi Group
10:30 – 11:00	**FIRESIDE CHAT** ▪ **Jennifer Carroll MacNeill** Minister of Health, Ireland ▪ **Marija Jakubauskienė** Minister of Health, Lithuania
11:00 – 11:10	**Nicolás González Casares** Member of the European Parliament (S&D, Spain) and member of Panel for the Future of Science and Technology (STOA)

Panel Discussions

11:10– 12:00	**POLICY DISCUSSION I** **Exploring new early access models to prioritise equitable access to innovative orphan therapies and diagnostics** Moderated by Josep Figueras, Director Emeritus of the European Observatory on Health Systems and Policies

	▪ **Stine Bosse** Member of the European Parliament (Renew, Denmark) ▪ **Peter Liese** Member of the European Parliament (EPP, Germany) ▪ **Alexis Arzimanoglou** European Reference Network for Rare and Complex Epilepsies (EpiCare) Coordinator ▪ **Silvio Brusaferro** Former President, Italian Institute of Health (2019-2023) ▪ **Alexandra Heumber Perry** CEO, Rare Diseases International ▪ **Johan Prevot** Executive Director, International Patient Organisation for Primary Immunodeficiencies (IPOPI)
12.00 – 12.15	**Olivér Várhelyi** European Commissioner for Health and Animal Welfare
12:15– 13:15	**Policy Session II** **Prioritising an EU Action Plan and Mission on Rare Diseases that unifies the rare disease stakeholder community** Moderated by Alexis Arzimanoglou, Coordinator ERN for Rare and Complex Epilepsies (EpiCare) ▪ **Avril Daly** President, EURORDIS-Rare Diseases Europe ▪ **Joanna Drake** DDG Planet, People and Science for Policy and Acting Director of the Clean Planet, DG RTD ▪ **Alexander Natz** Secretary General, EUCOPE ▪ **Nikos Papandreou** Member of the European Parliament (S&D, Greece) ▪ **Vlad Voiculescu** Member of the European Parliament (Renew, Romania) ▪ **Leonidas A. Phylactou** CEO and Medical Director, The Cyprus Institute of Neurology and Genetics ▪ **Birutė Tumienė** Co-chair ERN Board of Member States ▪ **Till Voigtländer** Coordinator JARDIN Joint Action
13:15 – 13:30	**Closing remarks** ▪ **Alexis Arzimanoglou** Coordinator ERN for Rare and Complex Epilepsies (EpiCare)

	▪ **Birute Tumiene** Co-chair ERN Board of Member States ▪ **Vytenis Andriukaitis** Member of the European Parliament (S&D, Lithuania) ▪ **Maurizio Scarpa** Director Regional Coordinating Centre for Rare Diseases, University Hospital Udine, Coordinator European Reference Network for Hereditary Metabolic Diseases (MetabERN) and Founder of Brains for Brain Foundation (Co-Chair)
13:30 - 14:30	*Signature of the **EU Declaration on Rare and Complex Diseases***

OUR SPEAKERS

Speaker biographies are presented in alphabetical order by surname within each day's programme.

DAY 1: RESEARCH & INNOVATION

Vytenis Andriukaitis

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

MEP Vytenis Andriukaitis is a Lithuanian politician, medical doctor and Member of the European Parliament. He previously served as Minister for Health of Lithuania (2012–2014) and as European Commissioner for Health and Food Safety (2014–2019). As European Commissioner, one of his major achievements was the establishment of the European Reference Networks, which advanced the care and treatment of patients with rare diseases.

MEP Andriukaitis is a co-author and signatory of the Independence Act of Lithuania (1990) and co-author of the Constitution of the Republic of Lithuania (1992). He was a founder of the Lithuanian Social Democratic Party, later serving as its Chairman (1999–2001) and is currently its Honorary Chairman.

Trained as a cardiovascular surgeon with over 20 years' experience, MEP Andriukaitis also holds a degree in History from Vilnius University. Since 2020, he has been Special Envoy of the WHO for Universal Health Coverage in the European region.

Richard Bergstrom

Vice President European Affairs, IQVIA

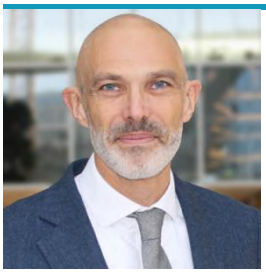
Richard Bergström is Vice President of European Affairs at IQVIA since September 2022. In June 2020, he was appointed to be Sweden's vaccine coordinator, and as such a member of the EU Joint Negotiation Team for Covid-19 vaccine contracts. He previously held the position as Director General of the European Federation of Pharmaceutical Industries and Associations (EFPIA) and served for nine years as the Director General of LIF, the Swedish Association of the Pharmaceutical Industry, following positions in Switzerland in regulatory affairs at the pharmaceutical companies Roche and Novartis. Mr. Bergström was also appointed by the Swedish Government to the Board of the Karolinska Institute.

Michael Berntgen

Head of Scientific Evidence Generation Department, EMA

Mr. Michael Berntgen is Head of the Scientific Evidence Generation Department at the European Medicines Agency (EMA) in Amsterdam. His department supports medicine development by ensuring robust and relevant scientific evidence, working with stakeholders such as patients and HTA bodies. Key activities include providing scientific advice, methodology qualification, support for paediatric and orphan medicines, and expertise in translational sciences. The department also manages the PRIME scheme, and fosters collaboration with decision-makers to support timely access to medicines.

A pharmacist with a PhD and a Master of Regulatory Affairs, Mr. Berntgen worked in regulatory affairs in Germany and the UK (1999–2006) before joining BfArM in Germany. In 2007 he moved to EMA, holding roles in anti-infectives and later leading therapeutic areas. He headed the Scientific and Regulatory Management Department (2013), then Product Development Scientific Support (2016). Since March 2020, he has led the Scientific Evidence Generation Department.



Niklas Blomberg

Executive Director, Innovative Health Initiative (IHI)

Dr. Niklas Blomberg serves as the Executive Director of the Innovative Health Initiative (IHI), a €2.4 billion public-private partnership between the European Union and European health industries focused on advancing collaborative health research across sectors such as pharmaceuticals, biotechnology, medical technology and digital health. He assumed this role in January 2024, bringing over twenty years of experience connecting life sciences research with data infrastructure and industry collaboration.

Prior to his current role, Dr. Blomberg was the Founding Director of ELIXIR, the European life sciences data infrastructure, where he established a pan-European network linking 23 member states and secured major EU-funded projects related to genomics, rare disease data and bioinformatics. He also spent fourteen years at AstraZeneca as a senior research scientist in respiratory and inflammation drug discovery, and co-led Open PHACTS, an Innovative Medicines Initiative project integrating drug discovery data to promote open science.

Dr. Blomberg holds a bachelor's degree in chemistry from the University of Gothenburg and a PhD in structural biology and bioinformatics from the European Molecular Biology Laboratory (EMBL) in Germany.

Emer Cooke

Executive Director of the European Medicines Agency

Emer Cooke has been the Executive Director of the European Medicines Agency, based in Amsterdam, since November 2020. She served as Chair of the International Coalition of Medicines Regulatory Authorities (ICMRA) for five years, from the beginning of her EMA mandate until the end of October 2025.

Between November 2016 and November 2020, she was the Director responsible for all medical product related regulatory activities at the World Health Organization in Geneva.

Ms Cooke is a pharmacist with Master's degrees in Science and Business Administration from Trinity College Dublin. She has over 30 years' experience in international regulatory affairs and held management positions at the EMA as Head of Inspections and Head of International Affairs respectively from 2002 until 2016.

Between 1998 and 2002 she worked in the Pharmaceuticals unit of the European Commission, where intra-alia, she was responsible for international collaboration, EU enlargement and the orphan medicines regulation. Prior to this, Ms Cooke worked at the European Federation of Pharmaceutical industries and Associations (EFPIA) (1992–1998), in the Czech Republic as an independent pharmaceutical policy advisor (1996–1998) and in the Irish pharmaceutical sector (1985–1990).

Starting her mandate as EMA's Executive Director in November 2020 amid a public health crisis of unprecedented scale she announced "My number one priority will be to drive forward EMA's response to the pandemic and the work already ongoing to support the development and approval of safe and effective COVID-19 vaccines and treatments." Doing precisely that, has earned her various awards including the 'European of the Year 2022' award by European Movement Ireland and Honorary Doctorates in Science (Royal College of Surgeons in Ireland – 2023 and National University of Ireland - 2025).

Jacques Demotes-Mainard

ECRIN Director General

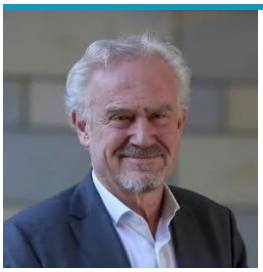
Prof. Jacques Demotes is the director general of the European Clinical Research Infrastructure Network (ECRIN), which he founded in 2004. In this role, he is responsible for the strategy and overall management of the infrastructure with the support of the ECRIN Management Office (located in Paris, France), the ECRIN European Correspondents (located in each Member / Observer country), and ECRIN's Scientific Partners (i.e., national networks of clinical trial units).

A neurologist and professor of cell biology, Prof. Demotes is an advisor to the biology and health research department at the French Ministry of Higher Education and Research. While at ECRIN, he has contributed to numerous initiatives and collaborative projects related to multinational clinical trials. In particular, he chaired the working group that

drafted the Organisation for Economic Co-operation and Development (OECD) Council Recommendation on the Governance of Clinical Trials.

Prior to ECRIN, he worked as a clinical neurologist and basic neuroscientist, then as director of the clinical investigation centre in Bordeaux.

Prof. Demotes received his MD (with a specialisation in neurology), a PhD in neuroscience, a Master of Science (MS) in neuroscience, and a Bachelor of Arts (BA) in mathematics and computer science from the University of Bordeaux. He also received an MBA from IAE Paris, and completed a training course in science policy with the Institute of Advanced Studies in Science and Technology in Paris.



Josep Figueras

Director Emeritus of European Observatory on Health Systems and Policies

Director Emeritus of European Observatory on Health Systems and Policies Josep Figueras, MD, MPH, PhD (Econ), FFPH, is Director Emeritus of the European Observatory on Health Systems and Policies and currently serves as Ambassador for the European Health Union Fellowships program. He was the founding director of the Observatory and, in addition to his work with the World Health Organization, has served major multilateral organizations such as the European Commission and the World Bank. He has served as a policy advisor in over forty countries across the European region and beyond.

Josep is a member of several governance, advisory, and editorial boards, including the Governing Board of the European Health Forum Gastein. His research focuses on health systems and policy, including comparative health systems, performance assessment, public health policy, and knowledge translation.

He is an Honorary Fellow of the UK Faculty of Public Health and has received numerous awards, including the Edward Kennedy Health Policy Award from Trinity College, the Spanish Ministry of Health Award, the European Health Forum Gastein Star, the Andrija Stampar Medal for Excellence in Public Health, and a Doctorate Honoris Causa from Semmelweis University.

Currently, he is a Visiting Professor at the London School of Economics and the NOVA National School of Public Health in Lisbon. He previously directed the MSc in Health Services Management and lectured at the London School of Hygiene & Tropical Medicine.

He obtained his PhD in health policy and financing from the University of London (LSE/LSHTM) and began his career as a specialist in Family and Community Medicine in Spain.

Holm Graessner

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

Dr. Holm Graessner is Managing Director of the Rare Disease Centre at the University and University Hospital Tübingen, a role he has held since 2010. He studied biomedical engineering, cybernetics, German language and literature, philosophy, business administration and marketing, obtaining a PhD (summa cum laude) in 2004 and an MBA in 2008.

He was a founding member of the first Centre for Rare Diseases in Germany, which established an umbrella infrastructure, therapy research centre and clinical information centre for rare diseases.

Dr. Graessner coordinates the European Reference Network for Rare Neurological Diseases (ERN-RND). Since 2020, he has been active in the European Academy of Neurology, serving on the management teams of the Neurogenetics Panel and the Rare Neurological Disease Coordinating Panel. He is also Commissioner for the Rare Diseases International Lancet Commission and recipient of the EURORDIS Leadership Award 2023.

Manuel Heitor

Former Portuguese Minister of Science, Technology, and Higher Education

Prof. Manuel Heitor served as Minister for Science, Technology and Higher Education of Portugal from 2015 to 2022, and previously as Secretary of State in the same field (2005–2011). He is Full Professor at Instituto Superior Técnico, Lisbon, where he earned his academic career after completing a PhD in Mechanical Engineering at Imperial College London and postdoctoral research at the University of California, San Diego. His research has focused on energy, environment, fluid mechanics and experimental combustion, alongside science, technology and innovation policies.

Prof. Heitor founded the IN+ Centre for Innovation, Technology and Development Policies at Instituto Superior Técnico in 1998, recognised internationally for its contributions to management of technology. He has held visiting positions at the University of Texas at Austin and Harvard University.

In 2023, the European Commission appointed him to chair the expert group shaping the future of Horizon Europe and the forthcoming FP10 research and innovation programme.

Ralf Dieter Hilgers

RealiseD Coordinator

Prof Dr Ralf-Dieter Hilgers studied mathematics at RWTH Aachen University. He finished his doctoral thesis at the statistical faculty of the University of Dortmund in 1991. In 2000 he received the Venia Legendi for Medical Statistics at the University of Cologne. Since 2001 he is head of the Department of Medical Statistics at the Medical Faculty, RWTH Aachen University. His research interest is in optimal design of experiments, randomizations

procedure and clinical trials. Since 1987 he gives biostatistical advice to clinical and experimental trials in all clinical and preclinical areas. Professor Hilgers teaches 300 students in different bio-scientific areas per year and is responsible for the education of investigators in clinical trials. He also acts as reviewer for methodological and clinical journals with main focus on surgical trials. From 2013 to 2017 he coordinated the FP7 funded IDeAl project (GA No. 602552). He co-lead from 2018 to 2024 WP20 of the H2020 funded EJP-RD (GA No. 825575) and from 2018 to 2026 WP4 of the ERICA-Project (GA No. 964908). Currently he is participating to ERDERA (GA No. 101156595) and coordinator of the IHI funded RealiseD (GA No. 101165912) project.

Virginie Hivert

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

Virginie Hivert is Acting CEO and Head of Therapies & Access at EURORDIS–Rare Diseases Europe. She drives the organisation's strategic work to advance the development of medicines for people living with rare diseases and to embed meaningful patient engagement across research, regulatory processes and access pathways. Since joining EURORDIS in 2014, she has been a leading figure in the organisation's collaboration with the European Medicines Agency, serving as an Observer on the Committee for Orphan Medicinal Products, as the PRAC Alternate member representing patient organisations, and now as a Civil Society representative on the EMA Management Board.

Internationally, Virginie has played an influential role in the International Rare Diseases Research Consortium since 2011, including four years as vice chair of its Therapies Scientific Committee. She brings more than 25 years of experience across research, healthcare and rare disease advocacy, supported by a PharmD and a PhD in Biological Sciences.

Arjon van Hengel

Senior Policy Officer, Health Innovations & Ecosystems, DG RTD

Dr. Arjon van Hengel works for the European Commission where he is currently a senior policy officer in the Health Innovations & Ecosystems Unit within the Directorate-General for Research and Innovation. This unit aims to foster the development and uptake of breakthrough innovations in the field of health and care, and to help citizens stay healthy in particular through health promoting environments and people-centred healthcare systems.

Mr. van Hengel has been at the European Commission since 2005 and has worked on various aspects of research related to human health. He has been team leader for infectious diseases during the first one and a half years of the COVID-19 pandemic, policy officer for antimicrobial resistance research and has led a research group that develops and validates analytical detection methods for food allergies.

Prior to joining the European Commission, he was a research scientist at the John Innes Centre (Norwich, UK). Mr. van Hengel studied biology at the University of Utrecht (NL) and received a PhD in molecular biology from the University of Wageningen (NL)

Sonata Jarmalaite

Adviser to the President of the Republic of Lithuania

Prof. Sonata Jarmalaite is the Adviser to the President of the Republic of Lithuania and the professor of Genetics at the Life Sciences Center of Vilnius University. She has a M.Sc. in Biology and Medical Biology, M.Sc. in Health management and Politics, and Ph.D. in Biology (Genetics).

For a decade, she served as Deputy Director for Research and Development at the National Cancer Institute of Lithuania. Her current research activities are mainly focused on translational cancer research and personalized medicine.

Prof. Jarmalaite was a committee member of the National Health Council and the Future Biomedicine Foundation, a national representative in EC Cancer sub-group and an expert of Horizon Europe the EU Cancer mission working group, served as the appointed representative for the Special 1+MG Group of the EU 1MG initiative.

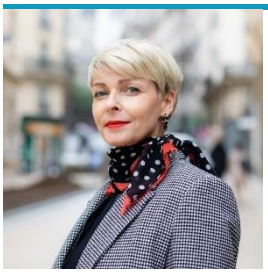
She is a recipient of the Lithuanian science prize, the Grand Duke Gediminas cross and St. Kristoforas statuette.

Adam Jarubas

Member of the European Parliament (EPP, Poland)

MEP Adam Jarubas is a Member of the European Parliament (EP) for the European People's Party (EPP), representing Poland. In the EP, MEP Jarubas has established himself as a key figure in health policy, currently chairing the European Parliament's Committee on Public Health (SANT) and previously involved in the Special Committee on Beating Cancer (BECA). He also serves on the Committee on Industry, Research and Energy (ITRE) and the Committee on the Environment, Public Health and Food Safety (ENVI). His focus areas include public health, green transformation, and digital transformation in the EU, where he coordinates the work of the Polish delegation in the EPP Group

MEP Jarubas obtained a PhD in social sciences and regional policies from Jan Kochanowski University in Kielce in 2018 and currently works as an Assistant Professor at the Institute of International Policy and Security. Between 2006 and 2018, he served as Marshal of the Świętokrzyskie Voivodeship, gaining extensive experience in regional health policy administration.



Daria Julkowska

European Rare Diseases Research Alliance (ERDERA) Coordinator

Daria Julkowska has a PhD in molecular biology and pursued her scientific vocation by the post-doctoral experience in cellular biology, at Institut Pasteur, Paris and extensive training in communication and European Union counselling. She also holds MSc in Management of Research from the University of Paris Dauphine. She coordinated the European Joint Programme on Rare Diseases and is currently the scientific coordinator of ERDERA rare diseases partnership that brings together over 180 institutions representing different type of stakeholders (researchers, funders, clinicians, industry & patients) from 37 countries from Europe and beyond. She is involved in the rare diseases field since 2010. She developed and put into action a set of collaborations facilitating research, including the partnerships with European Research Infrastructures, Patients' Organizations and industry. She has an extensive knowledge and understanding of European funding schemes and programmes and served as the chair of the Expert Group on support for the strategic coordinating process for European partnerships of the European Commission. She received the EURORDIS Black Pearl Award for European Rare Disease Leadership in 2020, and in 2024 she was again recognised with the Prix INSERM and the Remarkable Pole distinctions.

Hans Kluge

WHO Regional Director for Europe

Dr. Hans Henri P. Kluge began his second term as WHO Regional Director for Europe on 1 February 2025 after unanimous renomination.

A Belgian physician with over 30 years of global medical and public health experience, he began his career as a family doctor before serving with Médecins Sans Frontières in Liberia, Somalia and the Russian Federation, focusing on tuberculosis control in crises and prisons.

He joined WHO in 1999, holding roles in the Russian Federation and Myanmar and advising several Asian countries. Moving to the WHO Regional Office for Europe in 2009, he later led the Division of Health Systems and Public Health.

As Regional Director from 2020 to 2025, he guided the Region through COVID-19, mpox and conflict-related emergencies, while advancing digital health, behavioural insights, mental health and vaccine delivery. He launched major regional initiatives, including the Monti Commission and the Pan-European Network for Disease Control.

Ruth Ladenstein

European Reference Network for Paediatric Oncology (PaedCan) Coordinator

Prof. Ruth Ladenstein is Professor of Paediatrics and Senior Consultant in Paediatric Haematology and Oncology at the St. Anna Children's Hospital, Vienna. She heads the Clinical Trials Unit S2IRP (Studies & Statistics for Integrated Research and Projects) at the Children's Cancer Research Institute. Prof. Ladenstein also coordinates the European Reference Network for Paediatric Cancer (ERN PaedCan), as well as the Austrian Medicines for Children Research Network (OKIDS).

Prof. Ladenstein has played a leading role in European and international oncology networks. She is a board member of SIOP Europe, having served as its president (2009–2012) and co-founded the SIOPEN Association to foster neuroblastoma research.

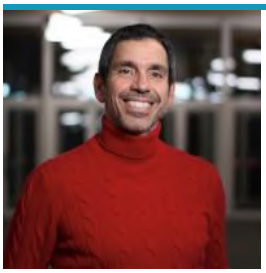
From 2019 to 2021, she was a member of the EU Mission Board for Cancer under Horizon Europe, advising the European Commission on optimising cancer research and innovation policy.

Enrico Letta

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

Enrico Letta served as Prime Minister of Italy from 2013 to 2014. Currently, he is the Dean of the IE School of Politics, Economics, and Global Affairs at IE University, President of the Jacques Delors Institute and President of AREL. In September 2023 the European institutions tasked him with the elaboration a Report on the Future of the Single Market. From March 2021 to February 2023, he was the National Secretary of the Democratic Party in Italy. Prior to that, from 2015 to 2021, he was Dean of the Paris School of International Affairs at Sciences Po Paris. In 2019, he was appointed President of the Association of Professional Schools of International Affairs (APSIA) for a two-year term. Earlier in his career, Letta served as Minister for EU Affairs (1998–1999), Minister for Industry, Trade and

Crafts (2000), Minister for Industry and Foreign Trade (2000–2001), and Undersecretary of State to Prime Minister Romano Prodi (2006–2008).



Georgios Margetidis

Head of Sector, EU4Health grants on cancer, NCDs, mental health, rare diseases and health workforce, HaDEA

Georgios Margetidis is an alumnus of the ENA in France.

He started his career in the Greek civil service, first steering important reforms in local government and health systems and then as CEO of the "Aghii Anarghiri" Cancer Center in Athens.

After joining the EC in 2006 he followed more than 150 projects spanning areas as diverse as eHealth and HTA, rare diseases and cancer, pharmaceuticals (pharmacovigilance, access and pricing), health systems, crisis preparedness.

Head of sector in the Health and Digital Executive Agency, since 2021, he has overseen more than EUR 1 billion of EU funding awarded under the EU4Health programme. His responsibilities include strategic planning, operational verification, budget execution, monitoring and reporting.

Mariska Mulder

VP Head of Regulatory Affairs EMEA and LATAM, Labelling & Marketed Products, Ultragenyx

Ms. Mariska Mulder has over 22 years of experience in Regulatory Affairs, spanning global and regional positions. Her expertise covers all drug development stages and life cycle management across diverse therapeutic areas, including infectious diseases (vaccines), rare diseases, immunology & inflammation, neurology, and oncology. She joined Ultragenyx in Nov 2022 as Head of Regulatory Affairs for EMEA, later expanding her role to include LATAM and global oversight for marketed products in Oct 2023 and global labelling in March 2025. Previously, Ms. Mulder held leadership roles at Sanofi, Novartis Vaccines & Diagnostics (now part of GSK vaccines), Crucell (now part of Janssen Vaccines) and Solvay Pharmaceuticals (now part of Abbott), focusing on different therapeutic areas and global business units. She holds an MSc and PharmD in Pharmaceutical Sciences from Utrecht University.

Alexander Natz

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

Dr. Alexander Natz serves as Secretary General of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE) in Brussels, representing innovative pharmaceutical and biotech companies towards the EU institutions, EMA and the relevant EU stakeholders and associations. He combines his EUCOPE leadership with his position

as Partner at Novacos Rechtsanwälte, where he advises innovative life sciences companies, including start-ups of the pharma and biotech sector, on European and German life sciences law covering pharmaceuticals, medical devices, food and cosmetics.

His legal expertise encompasses EU and German law perspectives on pharmaceutical regulation, having previously worked as Head of the Brussels Office of Bundesverband der Pharmazeutischen Industrie (BPI) from 2008-2013. Before, he worked as a lawyer at Sträter Law Firm in Germany with a special focus on managed entry agreements and licensing of pharmaceuticals. His career also included experience in competition law with the European Commission and pharmaceutical industry work. He conducted research on international pharmaceutical law as a research assistant at Duke University and earned his doctorate under supervision of former European Court of Justice judge Prof. Dr. Ulrich Everling.

Alberto Pereira

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

Alberto M. Pereira (Uruguay, 23 nov 1966) is Professor of Medicine and Head of the Department of Endocrinology and Metabolism, at the Amsterdam University Medical Center, the Netherlands. He was President of the European Neuroendocrine Association (2016-2018), and since 2017 he is the Coordinator of the European Reference Network on Rare Endocrine Conditions (Endo-ERN). He is also PI of the Horizon2020 ERICA project, that aims to strengthen the ERN's research and innovation capacity, and co-chair of the European Society of Endocrinology (ESE) Rare Disease Committee. His clinical and translational research focusses on the long-term effects of hypothalamic and pituitary diseases, and in specific on the effects of stress hormones on the brain. He has authored more than 390 peer-reviewed publications (H-index 68), and 12 book chapters (2 editorships). He is the recipient of two prestigious Awards, the ESE Clinical Endocrinology Trust Award (2022), and the Marcello Bronstein Mentorship in Pituitary Medicine Award (2025) from the Pituitary Society.

Enrico Piccinini

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

Enrico Piccinini brings a deep sense of purpose and decades of leadership to his role as Senior Vice President, Europe and International at Chiesi Global Rare Diseases. Since 2019, he has led efforts to expand access to therapies across Europe and emerging markets, ensuring that patients with rare diseases—regardless of geography—can benefit from innovative treatments and sustained support.

With a career rooted in specialty care and rare disease, Enrico previously held senior roles at Genzyme and Sanofi Genzyme, including General Manager for Italy and Malta, as well as broader leadership across EMEA and EU regions. He has successfully launched and

grown numerous therapies in complex markets, always with a commitment to ensuring impact reaches the patient level.

As an active member of several rare disease and pharma/biotech associations, Enrico is a passionate advocate for access, equity, and collaboration across borders. His global vision and patient-first approach continue to drive Chiesi's mission to serve the rare disease community with compassion and conviction.

Vinciane Pirard

Global Medical Affairs – Rare Diseases, Sanofi

Vinciane Pirard serves as the Rare Medical Scientific Advocacy Lead at Sanofi, where she plays a pivotal role in advancing scientific initiatives in the rare disease space. In this capacity, she bridges the critical gaps between scientific innovation, drug development, and evidence-based decision-making to accelerate availability of treatment options for patients with rare conditions.

With over 20 years of experience in both public and medical affairs, Vinciane chairs the International Rare Diseases Research Consortium (IRDiRC) Company Constituent Committee and actively contributes to several multi-stakeholder public-private partnerships, including RealiseD, fostering collaboration across the rare disease ecosystem. Her extensive expertise continues to drive meaningful impact.

Axel Pries

President, World Health Summit

Axel Radlach Pries is a German professor of physiology, President of the World Health Summit and former Dean of the Charité - Universitätsmedizin Berlin. After completing his medical degree and doctorate at the University of Cologne, he joined the Freie Universität Berlin, becoming a full professor and later Head of the Institute for Physiology at Charité (2001–2015). His research and leadership have focused on vascular biology and microcirculation, areas in which he has held numerous international roles, including General Secretary of the German and European Societies for Microcirculation and Chair of the International Liaison Committee for Microcirculation.

He also held leadership positions within the European Society of Cardiology and served as President of the Biomedical Alliance in Europe (2018–2019). From 2018 to 2020, he was interim CEO of the Berlin Institute of Health, and in 2023 he became Prorector for Medicine at the Danube Private University.

Maurizio Scarpa

Director Regional Coordinating Centre for Rare Diseases, University Hospital Udine, Coordinator European Reference Network For Hereditary Metabolic Diseases (MetabERN) and Founder of Brains for Brain Foundation (Co-Chair)

Maurizio Scarpa, MD PhD, paediatrician, is the Director of the Regional Coordinating Centre for Rare Diseases at the University Hospital of Udine, Italy. He is Professor of Paediatrics at the Dept. for the Woman and Child Health, University of Padova, Italy, and the Co-Founder of the Brains For Brain Foundation, together with Prof. David Begley, Kings College of London, London, UK. Prof. Scarpa has extensive expertise as a basic scientist in genetics and biotechnology, as well as a clinician in the diagnosis and treatment of paediatric rare disorders; neurometabolic diseases in particular. Together with dr. Christina Lampe he founded the Center for Rare Diseases at the Helios Dr. Horst Schmidt Kliniken in Wiesbaden, Germany. He is especially interested in developing innovative health approaches for the diagnosis and the treatment of metabolic inherited diseases; to this aim he is also collaborating with major biotech companies as an external independent expert. Prof. Scarpa is the Coordinator of the European Reference Network for Hereditary Metabolic Diseases, MetabERN, formed by 101 healthcare providers in 27 EU countries (www.metab.ern-net.eu) to facilitate patient-centered holistic activities to implement knowledge, diagnosis, management and treatment for inherited metabolic diseases.

Enrique Terol

Health Attaché to the Permanent Representation of Spain to the EU

Dr. Enrique Terol is Health Counsellor at the Permanent Representation of Spain to the European Union, representing Spanish interests in health policy and coordinating the health agenda. A medical doctor specialised in family and community medicine, he holds an MSc and PhD in Public Health, alongside further training in healthcare management and quality of care. His career spans clinical practice, hospital management and healthcare planning. Between 2004 and 2008, he served as Deputy Director General for Quality and Health Planning at the Spanish Ministry of Health, leading national strategies on ischaemic heart disease, diabetes, mental health, rare diseases and patient safety.

From 2011 to 2020, Dr. Terol was a Seconded National Expert and policy officer in the European Commission's DG SANTE, where he helped establish the European Reference Networks under the Cross-Border Healthcare Directive. He was later a team leader in the Commission's Medical Service unit, before returning to his current post in 2022.

Mark Turner

Chief Executive Officer, Conect4Children Stichting

Professor Mark Turner serves as Chief Executive Officer of Conect4Children Stichting, where he leads one of Europe's foremost initiatives dedicated to improving medicines for babies, children, and young people. As CEO since 2023, Professor Turner has guided the transition of this Dutch non-profit from a grant-funded phase under the Innovative Medicines Initiative (IMI2) to a sustainable organisation supporting clinical research for both academic and industry sponsors.

With more than thirty years of experience as a clinical neonatologist, Professor Turner is deeply committed to advancing paediatric drug development, particularly for rare diseases.

Throughout his career, Professor Turner has been actively involved in initiatives to improve paediatric medicines research, serving on the executive committee of the English Medicines for Children Research Network and as President of the European Society for Developmental, Perinatal, and Paediatric Pharmacology and chairing the European Network of Paediatric Research at the European Medicines Agency for six years. He has authored more than 300 publications and co-edited the leading textbook on paediatric drug development.

Professor Turner's leadership in research infrastructure and his collaboration with regulators, pharmaceutical industry, people with lived experience of ill-health, and research networks at national and international levels have advanced the design and conduct of high-quality paediatric clinical trials. He has also contributed to the development of methods for evaluating drug safety and medicines administration, with a particular emphasis on improving outcomes for children with rare and complex health conditions.

Sheela Upadhyaya

Together4Rare Steering Group Chair

Ms. Sheela Upadhyaya is a life sciences consultant and former lead of NICE's Highly Specialised Technology Programme for rare and ultra-rare disease treatments. She also served as NICE's strategic adviser for rare diseases and COVID-19. Before NICE, she commissioned rare disease services in the NHS, working closely with industry, clinicians, and patient groups.

She chairs Together for Rare Diseases (Together4RD), fostering collaboration between ERNs and industry, and co-chairs the HTAi Rare Disease Interest Group. She has co-authored several papers on HTA methods for orphan medicines and presented widely on the topic. At the European level, she has contributed to numerous rare disease policy and advocacy initiatives, campaigning for stronger infrastructure and better support for the rare disease community. She also collaborates extensively with patient groups and

policymakers in both the EU and UK to improve care and support for people living with rare diseases.

Aurelijus Veryga

Member of the European Parliament (ECR, Lithuania)

MEP Aurelijus Veryga is a psychiatrist and Professor at the Lithuanian University of Health Sciences with more than a decade of professional experience in addiction treatment. He co-founded the Kaunas Drug Abuse Help Centre for Youth and the Lithuanian National Tobacco and Alcohol Control Coalition, and has long been active in national health policy and prevention initiatives.

Between 2011 and 2016, he was Head of the Health Research Institute, a WHO Collaborating Centre under the Faculty of Public Health at the Lithuanian University of Health Sciences. From 2016 to 2020, he served as Minister of Health of Lithuania in the Skvernelis Cabinet.

In 2024, MEP Veryga was elected to the European Parliament, where he serves as a member in the Committee on the Environment, Climate and Food Safety, and a substitute in the Committee on Public Health. He is also a member of the WHO Regional Advisory Council on Innovation for Noncommunicable Diseases, advising on strategies to strengthen prevention and health promotion.

Marina Zanchi

Director of the European Health and Digital Executive Agency (HaDEA)

Marina Zanchi became Director of HaDEA in February 2022.

Ms. Zanchi joined the European Commission in 1992, and has served as Head of Unit in a variety of posts in the Directorate-General for Research and Innovation, working on issues including administration and finance, audit and the delegation of Research and Innovation Programmes to the Executive Agencies and Joint Undertakings and in the Directorate-General for Informatics working in the field of human resources.

Before joining HaDEA, Ms. Zanchi was the Head of the 'Future Society' Department in the European Research Executive Agency.

Prior to the Commission, Ms. Zanchi worked for the Food and Agriculture Organization of the United Nations and for the International Atomic Energy Agency.

DAY 2:
EU INFRASTRUCTURE & SKILLS

Teresa Ribera

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

Ms. Teresa Ribera is Executive Vice-President of the European Commission for a Clean, Just and Competitive Transition. She previously served as Vice-President of the Government of Spain and Minister for the Ecological Transition and the Demographic Challenge (2018–2023), where she played a leading role in shaping Spanish and European climate policy.

From 2014 to 2018, Ms. Ribera was Executive Director of the Institute for Sustainable Development and International Relations (IDDRI) in Paris, contributing to the negotiations of the Paris Climate Agreement. Earlier in her career, she was Spain's Secretary of State for Climate Change and Biodiversity (2008–2011) and held senior positions in the Ministry of the Environment and in the renewable energy sector.

A graduate in law from the Complutense University of Madrid, she also holds a diploma in constitutional law and political science from the Centre for Political and Constitutional Studies. She has taught public law at the Autonomous University of Madrid.

Christel Schaldemose

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

Ms. Christel Schaldemose has been a Member of the European Parliament (MEP) for Denmark, representing the Social Democrats (S&D), since 2006 and serves as Vice-President of the European Parliament since 2024. As Vice-President, among other responsibilities, Schaldemose chairs the Bureau Working Group on Digital Transformation, Cybersecurity and Information Security and replaces the EP's President in relations with G7, G20, EEA, EFTA countries, and Nordic/Baltic/Arctic nations. She is also a substitute member of the Committee on the Environment, Public Health and Food Safety (ENVI).

MEP Schaldemose's extensive parliamentary experience included serving as a member of the Special Committee on Artificial Intelligence, where she worked with, among other things, digital development, consumer protection and the green transition. She also gained significant recognition as the Parliament's rapporteur on the Digital Services Act (DSA) in 2021. She holds a master's degree in history from the University of Southern Denmark and previously worked in adult education and served as Secretary General of the Danish Adult Education Council.

Dariusz Adamczewski

Managing Director, Children's Tumor Foundation Europe

Dr. Dariusz Adamczewski is the Managing Director of Children's Tumor Foundation (CTF) Europe, where he provides strategic leadership to advance the Foundation's mission across the region. He represents CTF Europe with key stakeholders, including policymakers, regulators, industry partners and patient groups, while building partnerships to strengthen NF research and advocacy.

Dr. Adamczewski holds an MD in public health from the Medical University of Lodz and a Master of Public Administration from the National School of Public Administration in Warsaw. He brings over 20 years of experience in health policy, system coordination and EU advocacy. Most recently, he served as EMEA Director of Government Affairs and Policy at Johnson & Johnson in Brussels, leading healthcare policy engagement across oncology, cardiology, rare diseases and obesity. His prior leadership roles include Medical Director of the National Institute of Rehabilitation in Poland and Director at the Polish Ministry of Health.

Vytenis Andriukaitis

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

MEP Vytenis Andriukaitis is a Lithuanian politician, medical doctor and Member of the European Parliament. He previously served as Minister for Health of Lithuania (2012–2014) and as European Commissioner for Health and Food Safety (2014–2019). As European Commissioner, one of his major achievements was the establishment of the European Reference Networks, which advanced the care and treatment of patients with rare diseases.

MEP Andriukaitis is a co-author and signatory of the Independence Act of Lithuania (1990) and co-author of the Constitution of the Republic of Lithuania (1992). He was a founder of the Lithuanian Social Democratic Party, later serving as its Chairman (1999–2001) and is currently its Honorary Chairman.

Trained as a cardiovascular surgeon with over 20 years' experience, MEP Andriukaitis also holds a degree in History from Vilnius University. Since 2020, he has been Special Envoy of the WHO for Universal Health Coverage in the European region.

Kim Angel

Executive Director of the International Mucopolysaccharidosis Network

Kim Angel is the Executive Director of the International MPS Network (IMPSN) and a committed advocate for individuals and families affected by mucopolysaccharidoses (MPS) and related rare diseases. With a background in organizational leadership and a deep passion for driving meaningful change, Kim has been instrumental in advancing global collaborations, strategic initiatives, and impactful programs that promote access to care, innovative research, and support services for the MPS community. Her visionary leadership continues to shape the future of the IMPSN and the broader rare disease landscape, uniting

voices worldwide to ensure a better quality of life for all those impacted by MPS. Kim was the Executive Director of the Canadian MPS Society from 2016 to 2024. Under her leadership, the Canadian MPS Society has seen significant growth in its programs and services, expanded its advocacy efforts, and enhanced its support for individuals and families affected by mucopolysaccharidoses (MPS) across Canada.

Michael Berntgen

Head of Scientific Evidence Generation Department, EMA

Mr. Michael Berntgen is Head of the Scientific Evidence Generation Department at the European Medicines Agency (EMA) in Amsterdam. His department supports medicine development by ensuring robust and relevant scientific evidence, working with stakeholders such as patients and HTA bodies. Key activities include providing scientific advice, methodology qualification, support for paediatric and orphan medicines, and expertise in translational sciences. The department also monitors the human medicines portfolio, manages the PRIME scheme, and fosters collaboration with decision-makers to support timely access to medicines.

A pharmacist with a PhD and a Master of Regulatory Affairs, Mr. Berntgen worked in regulatory affairs in Germany and the UK (1999–2006) before joining BfArM in Germany. In 2007 he moved to EMA, holding roles in anti-infectives and later leading therapeutic areas. He headed the Scientific and Regulatory Management Department (2013), then Product Development Scientific Support (2016). Since March 2020, he has led the Scientific Evidence Generation Department.

Szymon Bielecki

Head of Sector, AI health tech and infrastructure, DG CONNECT

Szymon Bielecki is Head of Sector at the European Commission, Directorate-General for Communications Networks, Content and Technology (DG CNECT). He works on the design and implementation of actions related to health data infrastructures for research, innovation, personalised medicine and public health under Digital Europe and other funding schemes. Among others, he supervises DG CNECT's support for the European 1+ Million Genomes initiative and for actions fostering health data re-use, e.g. related to the European Health Data Space or application of Artificial Intelligence in the health sector.

James R. Bonham

President of the International Society of Neonatal Screening

Prof. James R. Bonham serves as President of the International Society of Neonatal Screening (ISNS), which represents more than 450 members across 84 countries. He earned his PhD and trained in clinical biochemistry, dedicating his career to newborn screening for early detection of inherited metabolic diseases. Prof. Bonham worked as Clinical Director at Sheffield Children's Hospital from 1992 until 2018 and serves since 2015 as the UK National Laboratory Advisor for the Newborn Screening Blood Spot Programme with NHS England.

He is also an adviser to the National Screening Committee on behalf of Department of Health and Social Care. In recognition of his outstanding contributions to young people with genetic metabolic diseases, he was awarded an MBE in 2019.

Christophe Clergeau

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

MEP Christophe Clergeau is a French politician with the Socialist Party. He sits as a member on the Committees for Environment, Public Health and Food Safety and the Subcommittee on Public Health.

Before entering the European Parliament, MEP Clergeau built an academic and political career. He was a regional councillor for Pays de la Loire from 2004, serving as vice-president of the regional council between 2009 and 2015, with responsibility for economic development and innovation. From 1997 to 1998, he headed the cabinet of the French Minister for Agriculture and Fisheries, Louis Le Pensec, after earlier roles at Radio France Internationale. He also served as national secretary for Europe for the Socialist Party since 2019 and chaired the party's Pays de la Loire regional federation since 2009.

Alain Coheur

President of the Industrial Change, European Economic and Social Committee

Mr. Alain Coheur is a member of the European Economic and Social Committee since 2015, where he is currently President of the Consultative Committee on Industrial Change (CCMI). Between 2022-2023, he was President of the Section for the Single Market, Production and Consumption (INT).

In 2022, Mr. Coheur served as the Rapporteur for the EESC Opinion on rare diseases, recommending establishing a comprehensive European action plan on rare diseases with SMART goals attainable by 2030. In 2024, he was a recipient of the EURORDIS Policy Maker Award.

During the main part of his career, Mr. Coheur was in charge of various positions in the development of social economy organizations. He is currently the Director for European and International Affairs at Solidaris, the Belgian National Union of Socialist Mutual Health Funds (since 2002). In 2013, he was appointed by the European Commission as a member of the consultative multistakeholder group on social business (GECES). He was elected President of the Belgian Non-Governmental Organisation "Solsoc" in 2007 and President of the European platform "Social Economy Europe" in 2008.

Jane Cooper

Senior Vice-President, Head of EMEA, Ultragenyx

Jane leads Ultragenyx's regional strategy, development, operations and commercialisation across Europe, The Middle East and Africa region, driving patient access to innovative therapies for rare and ultra-rare diseases.

Prior to this role, Ms. Cooper held several leadership positions at Biogen from April 2012 to August 2022, including Head of Neuro Muscular Diseases for the Biogen Intercontinental Region and SMA Regional Lead for International Partner Markets. Her responsibilities encompassed market development, regulatory affairs, commercialization, and operations across numerous countries.

Previous experience includes an Associate Principal role at McKinsey & Company, where Ms. Cooper advised Fortune 500 healthcare and technology firms, and a Partner position at CSC Index, supporting clients across diverse sectors. She began their career in sales and marketing with Butterfield & Robinson and had a foundational role at TD Bank Group in corporate finance.

Ms. Cooper holds multiple degrees, including an MBA in Finance and Strategy from London Business School and a BSc in Pre-Med and Economics from the University of Otago.

Hélène Dollfus

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

Prof. Hélène Dollfus is a consultant at the University Hospital of Strasbourg (HUS) with dual specialisation in clinical genetics and ophthalmology, and Professor of Medical Genetics at the University of Strasbourg. She heads the HUS Medical Genetics Department and the CRMR CARGO reference centre for rare eye diseases, and leads the Laboratory of Medical Genetics (UMRS-1112, Inserm–University of Strasbourg).

Her research focuses on ultra-rare genetic diseases affecting the eyes and other organs, particularly ciliopathies. She has contributed to identifying over a dozen genes responsible for syndromes such as Bardet-Biedl, leading both clinical research (COBBALT cohort) and cellular studies to uncover disease mechanisms and therapeutic avenues. She also leads European projects on quality of life in visually impaired children (SeeMyLife) and AI for retinal dystrophy image analysis (RaReTiA).

Committed to improving patient care, she has shaped national rare disease strategies and genomic medicine in France. Since 2017, she has coordinated ERN-EYE that unites 60+ hospitals to advance rare eye disease care and research.

Teresinha Evangelista

European Reference Network for neuromuscular diseases (ERN EURO-NMD) Coordinator

Dr. Teresinha Evangelista is a neurologist with more than 20 years of clinical and research experience at the Neurosciences Department of Hospital de Santa Maria in Lisbon. Alongside her clinical work, she was a member of the Neuromuscular Research Unit at the Institute of Molecular Medicine and an Invited Lecturer at the Faculty of Medicine of the University of Lisbon.

After completing post-graduate training in neuropathology and working at INSERM in Paris, she established a fully equipped Neuromuscular Laboratory in Lisbon, which became a reference centre for southern Portugal and helped expand national neuromuscular diagnostic capacity.

She also served as President of the Portuguese Society for the Study of Neuromuscular Diseases and was a member of the national coordinating centre for lysosomal disorders. Her current work focuses on hereditary muscle diseases, FSHD and congenital myasthenic syndromes.

As part of EUCERD Joint Action and RD-Action, she helped conceptualise and establish the EURO-NMD ERN. She has authored over 70 publications.

Alessandra Ferlini

University of Ferrara, Italy & Scientific Coordinator of the EU-IHI Screen4Care project

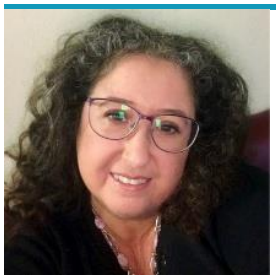
Professor Alessandra Ferlini is the Scientific Coordinator of Screen4Care, an EU-IHI funded research project involving a PPP consortium composed by 41 partners and dedicated to accelerating the diagnosis of rare diseases through genomic newborn screening and digital health innovation.

She is Professor of in Medical Genetics and Head of the Medical Genetics Unit at the University of Ferrara, Italy, also holding an honorary visiting professorship at University College London. Following several clinical and research experience in Italy (Nobel prize Dulbecco Team in Milan) and abroad, Professor Ferlini received her PhD in London at the Imperial College of Medicine.

Professor Ferlini has coordinated several European research grants in the field of rare diseases and played leading roles in international initiatives, including serving as Chair in international Working Groups, Boards and Committees, as ERN representative, and being PI of clinical trials for Duchenne muscle dystrophy. Her expertise spans genetic diagnosis, gene discovery, functional genomics, and the development of novel molecular therapies for muscular dystrophies.

As Screen4Care Coordinator, Professor Ferlini leads efforts across 21 academic and 19 industrial partners to shorten the diagnostic odyssey for rare disease families, piloting next-generation sequencing-based newborn screening for 245 genetic disorders in throughout

Europe. Her work continues to drive innovation in early diagnosis and personalized medicine for children with rare diseases.



Ilaria Galetti

Vice-President Federation of European Scleroderma Associations & ePAG ReCONNET

Ms. Ilaria Galetti is Vice-President of the European Federation for Systemic Sclerosis (FESCA) and of the Italian Association for Systemic Sclerosis (GILS), after years of volunteering at both national and European level.

Since 2017, Ms. Galetti has been the ePAG representative for systemic sclerosis within ERN ReCONNET, where she also co-chairs the Research and Quality of Care Working Group with Dr Rosaria Talarico of AOU Pisana, Italy. She is an alumna of the EURORDIS Summer and Winter Schools on research and clinical trials, the ISS School on Rare Disease Registries, and the Italian EUPATI Patient Academy.

Through her work, Ms. Galetti continues to advocate for equal access to care and research opportunities for rare disease patients, ensuring that the patient perspective is central to European policy and clinical practice.

Liesbet Geris

Professor Biomedical Engineering, Université of Liège and KU Leuven; Executive Director VPH Institute

Professor Liesbet Geris is a leading figure in biomedical engineering, holding professorial appointments at both the University of Liège and KU Leuven in Belgium. She currently serves as Executive Director of the Virtual Physiological Human Institute (VPH Institute), where she is a passionate advocate for the integration of in silico modelling within healthcare research, clinical translation and regulatory policy. Professor Geris is renowned for her pioneering work in multi-scale and multi-physics computational tissue engineering, engaging closely with clinical and industrial collaborators to develop innovative solutions for skeletal pathologies, regenerative medicine, and the optimisation of biomedical implant manufacturing.

As scientific coordinator of the Prometheus Platform for Skeletal Tissue Engineering and leader of major European research consortia, Professor Geris has been recognised with prestigious ERC grants and industry awards for her contributions to tissue engineering and computational modelling. In her capacity as executive director of the VPH Institute, Professor Geris fosters collaboration across academia, industry, patient organisations and regulatory authorities such as the European Medicines Agency (EMA) and the United States Food and Drug Administration (FDA). Her leadership in the in silico and digital medicine field continues to advance the understanding and treatment of rare, complex, and chronic diseases, bringing interdisciplinary teams together to accelerate the development and validation of digital health tools and fostering the next generation of biomedical innovators.

Jens K. Habermann

Director General, European Research Infrastructure for biobanking and biomolecular resources (BBMRI-ERIC)

Prof. Jens K. Habermann, M.D., PH.D., is Director General of the Biobanking and Biomolecular Resources Research Infrastructure – European Research Infrastructure Consortium since September 2020.

He is on leave of absence from the University of Lübeck as Head of the Section of Translational Surgical Oncology and Biobanking and as Head of ICB-L (Interdisciplinary Center for Biobanking-Lübeck).

Prof. Habermann obtained his M.D. training at the Medical University of Lübeck, Germany. He received his Ph.D. at the Cancer Center Karolinska, Karolinska Institute, Sweden and a Postdoctoral Fellowship at the National Cancer Institute, NIH, USA.

As board certified specialist in human genetics, Prof. Habermann combines clinics, biobanking and translational (cancer) research to optimize precision medicine.

Konstantinos Kleovoulou

Director of Research and Innovation, Deputy Ministry of Research, Innovation and Digital Policy, Cyprus

Dr. Konstantinos Kleovoulou is the Director of Research and Innovation at the Deputy Ministry of Research, Innovation and Digital Policy of the Republic of Cyprus. He coordinates the formulation and implementation of national policies on research and innovation, supporting the growth of a dynamic and competitive innovation ecosystem.

With prior leadership experience at The Cyprus Institute, a track record in private-sector innovation and entrepreneurship, and a research background at MIT and the Cyprus University of Technology, he bridges policymaking, business, and science. He serves on several national and European boards, where he advances research and innovation agendas with an emphasis on driving green and digital transitions.

Dr. Kleovoulou holds a Ph.D. in Physics and an MBA from University College London.

Ricardo Baptista Leite

CEO, HealthAI

Dr. Ricardo Baptista Leite is a Portuguese-Canadian medical doctor specialized in infectious diseases, with extensive experience in global health, health systems and science-based policymaking. He is the CEO of HealthAI - The Global Agency for Responsible AI in Health, based in Geneva and the founder and President of UNITE Parliamentarians Network for Global Health, bringing together policymakers from over 110 countries.

A former Member of Parliament in Portugal, he served on both the Health and Foreign Affairs Committees and is currently a City Councillor in Sintra. Dr. Baptista Leite is also Chair of the

Board of the Harvard-Charité Global Health Policy Lab in Berlin and Chair of the Centre for Global Health at NOVA IMS in Lisbon.

Trained at Johns Hopkins University and Harvard University, he has worked as a physician in Portugal's National Health Service and volunteered in humanitarian missions during the COVID-19 pandemic and the war in Ukraine.

Michael Marmot

Director of the Institute of Health Equity

Professor Sir Michael G. Marmot CH is Professor of Epidemiology at University College London and Director of the UCL Institute of Health Equity. For over four decades, he has led pioneering research into the social determinants of health and health inequalities, notably through the Whitehall II Study and the English Longitudinal Study of Ageing.

Professor Marmot chaired the WHO Commission on Social Determinants of Health, producing the landmark "Closing the Gap in a Generation report" (2008), and led the UK Government's "Fair Society, Healthy Lives" review (2010), widely known as the Marmot Review. His follow-up reports, including "Marmot Review 10 Years On" (2020) and "Build Back Fairer: COVID-19 Marmot Review (2020)", continue to shape policy in the UK and globally.

Internationally, he has chaired health inequality commissions across Europe, the Americas and the Eastern Mediterranean. Knighted in 2000 and appointed Companion of Honour in 2023, Professor Marmot has received numerous global awards and 21 honorary doctorates.

Martin McKee

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

Prof. Martin McKee is Professor of European Public Health at the London School of Hygiene and Tropical Medicine. He qualified in medicine in Belfast, Northern Ireland, with subsequent training in internal medicine and public health.

He was founding director of the European Centre on Health of Societies in Transition, a WHO Collaborating Centre, and which he led for over a decade. He is also research director of the European Observatory on Health Systems and Policies. He was chair of the Scientific Advisory Board of the Pan European Commission on Health and Sustainable Development (Monti Commission) and elected President of the British Medical Association for 2022/23. Prof. McKee is a former Chair of the UK Society for Social Medicine and Past President of the European Public Health Association.

He has published over 1,450 scientific papers and 50 books. His contributions to European health policy have been recognized by the award of honorary doctorates from Hungary, The Netherlands, Sweden, Greece, and visiting professorships at the Universities of Zagreb and Belgrade, the London School of Economics and Taipei Medical University. In 2003, he was

awarded the Andrija Stampar medal for contributions to European public health and in 2005 was made a Commander of the Order of the British Empire (CBE).

Donata Meroni

Head of Unit B3 'Health monitoring and cooperation, Health networks', European Commission

Donata Meroni is Head of the Unit 'Health monitoring and cooperation, Health networks' dealing with rare diseases, cross border healthcare policies, health information and Scientific Committees, in the Public Health Directorate of DG SANTE, European Commission. She was previously Head of the Unit dealing with health Promotion and diseases prevention and funding instruments, and head of the 'health and food safety' Unit in the Executive Agency for Consumers, Health, Agriculture and Food (CHAFEA) in Luxembourg.

She has been working with the Commission since 2002. Before, she had worked in Italy for 13 years in both private and public sector.

Nathalie Moll

Director General of the European Federation of Pharmaceutical Industries and Associations (EFPIA)

Ms. Nathalie Moll holds an Honours Degree in Biochemistry and Biotechnology from St Andrews University, Scotland and has since worked 30 years for the biotech and pharma industries at EU and national level in corporate and association positions.

She has been the Director General of the European Federation of Pharmaceutical Industries and Associations (EFPIA) since 2017. EFPIA represents the pharmaceutical industry operating in Europe. Together with its direct membership 40 leading pharmaceutical companies, 33 national associations representing over 1,900 companies and in collaboration with health and research players, EFPIA's mission is to create an environment that enables our members to innovate, discover, develop and deliver new therapies and vaccines for people across Europe, as well as contribute to the European economy. EFPIA was named Best Business EU Trade Association in Brussels in 2022.

Prior to her current role at EFPIA, Ms. Moll served at EuropaBio, lastly as Secretary General for 7 years, during which time the association was ranked most effective trade association in Brussels in 2013.

In 2013, Ms. Moll won the Technovisionaries Women Innovation Award organised by Women & Technologies® while in 2009, Nathalie and the Green Biotech Team of EuropaBio were presented with the Leadership and Excellence in Advancing Ag-Biotech and Food Issues Award. She was also named one of the 15 leading women in biotech in Europe in 2017, recognised as one of the "20 Women Who Shape Brussels" by POLITICO Europe in 2020 and Among the Women who advance associations in 2021.

Marta Mosca

European Reference Network of Connective Tissue and Musculoskeletal diseases (ERN ReCONNET) Coordinator

Dr. Marta Mosca is a professor of rheumatology at the University of Pisa and head of the Rheumatology Unit at the Azienda Ospedaliero Universitaria Pisana, Italy. She is also head of the Fellowship Training Program in Rheumatology at the University of Pisa and a faculty member of the Ph.D. program in Genetics, Oncology and Clinical Medicine of the Consortium comprising the Universities of Pisa, Florence and Siena.

She has participated in the development of European Alliance of Associations for Rheumatology (EULAR) recommendations in several different areas and has coordinated the development of recommendations for monitoring systemic lupus erythematosus (SLE) patients in clinical practice and in observational studies. She was also a member of the EULAR/American College of Rheumatology steering committee for the development of classification criteria for SLE. Dr. Mosca is a member of the scientific advisory board of LUPUS EUROPE, the European Association of SLE patients.

Since December 2016, she has been the coordinator of the European Reference Network on Rare and Hereditary Connective and Musculoskeletal Diseases (RECONNET). She has authored over 200 ISI publications on SLE and other connective-tissue diseases.

Peter Mulders

European Reference Network for Rare Uro-Recto-Genital Diseases and Complex Conditions (EUROGEN) Coordinator

Prof. Peter Mulders is an expert in the field of general and oncological urology. He conducts research both in the laboratory and the clinical settings. His research focuses in particular on prostate and kidney cancer. His primary activities include urological patient care, teaching and research in a multidisciplinary setting.

Prof. Mulders is head of the Urology Department and co-director of the Centre of Oncology at the Radboud Institute for Molecular Life Sciences, Netherlands.

Ciarán Nicholl

Head of the Health in Society Unit, Joint Research Centre

Dr. Ciarán Nicholl obtained his PhD in cancer research at Heidelberg University, an MSc at Kingston University and King's College Hospital, and a BSc in Chemistry at Galway University.

He joined the European Commission's Joint Research Centre (JRC) in 1995 as a postdoctoral fellow before moving into JRC communications, and since 2012 has focused on non-communicable diseases, particularly cancer and rare diseases.

Since 2013, Dr. Nicholl has led the JRC's Health in Society Unit in the Directorate for Health, Consumers and Reference Materials. With a team of 40, the unit operates the European Commission Knowledge Centre on Cancer, which brings together expertise through five

strategic pillars: the Health Promotion and Disease Prevention Knowledge Gateway, the European Cancer Information System, the Cancer Inequalities Registry, European guidelines and quality assurance schemes for cancer screening, and the EU Platform for Rare Diseases Registration

Michael Ostland

Head of Development Europe, Denali Therapeutics

Mr. Michael Ostland is Head of Development Europe at Denali Therapeutics, based in Zurich, a position held since 2022. With over two decades of experience in the biopharmaceutical industry, he has led global development strategies across multiple therapeutic areas, from early clinical research to commercialization.

Previously, Mr. Ostland spent over 20 years at Roche and Genentech in a variety of leadership roles. As Lifecycle Leader at Roche, he led cross-functional teams for kidney and rheumatologic disease portfolios, and for four years directed the team that developed and launched the first oral therapy for spinal muscular atrophy.

Mr.Ostland holds a PhD in Statistics from the University of California, Berkeley and a BA in Mathematics from UC San Diego.

Mar Mañu Pereira

Scientific Coordinator of European Reference Network on Rare Hematological Diseases

Dr. María del Mar Mañu Pereira serves as Scientific Coordinator of the European Reference Network on Rare Hematological Diseases (ERN-EuroBloodNet) and Head of the Rare Anaemia Disorders Laboratory at the Vall d'Hebron Institut de Recerca (VHIR) in Barcelona since 2018. She holds a PhD in Biosciences from the University of Barcelona and has over 20 years of experience in rare anaemia research. Dr. Mañu Pereira coordinates the European platforms for patient registries in haematology (ENROL) and rare anaemia disorders (RADeep), playing a crucial role in advancing diagnosis and treatment of rare blood disorders across Europe.

Her research focuses on integrative diagnosis of rare anaemia disorders, combining conventional and innovative methodologies including x-omics for personalised medicine. Before joining VHIR, Dr. Mañu worked for 14 years at the Red Cell Unit of Hospital Clínic i Provincial and 1,5 years at the Fundación Josep Carreras contra la Leucemia, where she specialised in diagnosing rare anaemias, genetic analysis of haemoglobinopathies, and served as Scientific Coordinator of the European Network on Rare and Congenital Anemias (ENERCA).

Martine Pergent

President, International Patient Organisation for Primary Immunodeficiencies (IPOPI)

Martine Pergent is President of the International Patient Organisation for Primary Immunodeficiencies (IPOPI), a global advocacy group representing patients with primary immunodeficiency disorders. She has held this position since October 2018, having previously spent eight years as IPOPI Vice-President and served on the board since 2004. Ms Pergent is also co-founder of IRIS, the French patient organisation for primary immunodeficiency, and remains closely involved with various national and global patient advocacy initiatives.

As President, Ms Pergent is actively engaged in IPOPI's strategic planning, advocacy, data collection, and stakeholder cooperation at international, European and national levels. She is particularly committed to raising awareness of primary immunodeficiencies (PIDs) as rare conditions, improving access to early and accurate diagnosis and treatment—including the global availability of immunoglobulins—and strengthening patient registries and epidemiology. Ms Pergent has led efforts to support the creation of patient organisations in low- and middle-income countries, and has championed the use of digital communications and e-health to benefit the rare disease community.

Ms Pergent's advocacy work is distinguished by her insistence that patients be recognised as essential partners in all policy, research and healthcare decisions that affect their lives. Her leadership continues to focus on improving outcomes, ensuring patient voices are both heard and heeded, and supporting the global rare disease community to achieve better, more equitable health for all those living with PIDs.

Ana Rath

Orphanet Director, INSERM

Dr. Ana Rath is a medical doctor with a background in general surgery and a Masters degree in Philosophy. She oriented her career to medical information and terminologies in 1997 and joined Orphanet in 2005, where she was Manager of the Orphanet Encyclopaedia, then Scientific Director, and Director of Orphanet and Coordinator of the Orphanet network since 2014.

Dr. Rath was the coordinator of RD-ACTION, the EU Joint Action for rare diseases (2015-2018) and of the International Rare Diseases Research Consortium (IRDiRC)'s Scientific secretariat until 2017. She chairs the Orphanet Rare Disease Ontology (ORDO), and was member of the WHO's ICD11 Revision Steering Committee.

She also coordinates projects on implementation of rare diseases codification in EU member states (RD-CODE and currently OD4RD) and co-chairs the ERDERA Data Service Hub on services around interoperable data and resources for RD research. She has responsibilities in JARDIN Joint Action focused on harmonisation of RD data in health systems.

Saila Rinne

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

Saila Rinne is the Head of Unit "Artificial Intelligence in Health and Life Sciences" at the EU AI Office in the Directorate-General for Communications Networks, Content and Technology (DG CONNECT) of the European Commission. Her team deals with EU policies and investments that foster innovative digital technologies and capacities in healthcare and life sciences. These include genomics and medical imaging data infrastructures, virtual human twins and Artificial Intelligence solutions for prevention, diagnostics and care. Ms. Rinne has had a long career of over 25 years at the Commission. She has previously worked on data policy and innovation projects in DG CONNECT and as a translator and terminologist in DG Translation.

Tamsin Rose

Professional Moderator

A political science graduate (1987-1990), Tamsin has been a radio journalist and communications expert. She managed an EU funding programme to twin cities in the EU and former Soviet Union. From 2002 to 2006, Tamsin served as Secretary-General of the European Public Health Alliance, a pan-European platform of NGOs. An independent consultant, Tamsin works with NGOs, institutions and philanthropic foundations on advocacy and organisational development. She helps clients see the bigger picture and develop fresh insight into the way that they work. As a former radio journalist, Tamsin trains people to speak confidently and effectively. She is also a Senior Fellow at Friends of Europe.

Luca Sangiorgi

European Reference Network on Rare Bone Diseases (ERN-BOND) Coordinator

Prof. Luca Sangiorgi is Director of the Department of Rare Skeletal Diseases at the Rizzoli Orthopaedic Institute in Bologna. Since 2017, he has coordinated the European Reference Network on Rare Bone Disorders (ERN-BOND) and currently chairs the ERN Coordinators Group, the governing board of all 24 ERNs. He is also Head of the Medical Genetics Department and coordinator of the Rizzoli Rare Disease Centre, which manages several national rare disease registries.

Prof. Sangiorgi earned his medical degree and oncology board certification at the University of Bologna and a PhD in Clinical Genetics at Sapienza University of Rome. His international experience includes a research fellowship at the US National Cancer Institute in Bethesda.

He has contributed to more than 100 peer-reviewed publications, and has coordinated or partnered in over 20 international research projects.

Maurizio Scarpa

Director Regional Coordinating Centre for Rare Diseases, University Hospital Udine, Coordinator European Reference Network For Hereditary Metabolic Diseases (MetabERN) and Founder of Brains for Brain Foundation (Co-Chair)

Maurizio Scarpa, MD PhD, paediatrician, is the Director of the Regional Coordinating Centre for Rare Diseases at the University Hospital of Udine, Italy. He is Professor of Paediatrics at the Dept. for the Woman and Child Health, University of Padova, Italy, and the Co-Founder of the Brains For Brain Foundation, together with Prof. David Begley, Kings College of London, London, UK. Prof. Scarpa has extensive expertise as a basic scientist in genetics and biotechnology, as well as a clinician in the diagnosis and treatment of paediatric rare disorders; neurometabolic diseases in particular. Together with dr. Christina Lampe he founded the Center for Rare Diseases at the Helios Dr. Horst Schmidt Kliniken in Wiesbaden, Germany. He is especially interested in developing innovative health approaches for the diagnosis and the treatment of metabolic inherited diseases; to this aim he is also collaborating with major biotech companies as an external independent expert. Prof. Scarpa is the Coordinator of the European Reference Network for Hereditary Metabolic Diseases, MetabERN, formed by 101 healthcare providers in 27 EU countries (www.metab.ern-net.eu) to facilitate patient-centered holistic activities to implement knowledge, diagnosis, management and treatment for inherited metabolic diseases.

Alberta Spreafico

Senior Vice President Health Innovation, EVERSANA; President, Innovation for Global Health Institute

Alberta Spreafico is a global health innovation pioneer and a leading thought leader in digital health policy and systemic health innovation strategies. As the Senior Vice President of Health Innovation at Eversana, she drives global health innovation initiatives, collaborating closely with stakeholders in the health and innovation ecosystems. Bridging research and practice, Ms. Spreafico serves as Adjunct Professor of Policy & Economics at the University of Pavia and Academic Fellow at SDA Bocconi, specializing in Health Policy and Health Innovation Research and Education.

She directs the Digital Health Policy Summit at Frontiers Health, is a Policy Knowledge Partner for the Digital Therapeutics Alliance and an External Advisory Board Member for the "HealthData@EU" project. Ms. Spreafico's extensive experience includes founding global health development programs in emerging economies, contributing to NASA's Benefits to Humanity and participating in multiple United Nations expert meetings. She is also a TEDx speaker, focusing on digitally enhancing health for all.

Tomislav Sokol

Member of European Parliament (EPP, Croatia)

Tomislav Sokol is a Croatian politician and Member of the European Parliament (MEP) since 2019. A member of the Croatian Democratic Union (HDZ), he plays a leading role in EU health policy and currently serves on the Subcommittee on Public Health. Notably, he negotiated the European Health Data Space (EHDS) on behalf of the European Parliament.

Before joining the European Parliament, MEP Sokol was Assistant Minister in the Ministry of Science and Education and later served as a Member of the Croatian Parliament. In Brussels, he has been active on the Special Committees on Beating Cancer (BECA) and Covid-19 (COVI) and hosted the INTERACT-EUROPE launch, part of Europe's Beating Cancer Plan.

He holds an LL.M. (2009) and a PhD (2014) from KU Leuven, focusing on EU health law and cross-border healthcare. He graduated in law magna cum laude from the University of Zagreb in 2006.

Anton Ussi

Operations and Finance Director, EATRIS

Mr. Anton Ussi is Operations & Finance Director for EATRIS C&S. He has a background in engineering and SME administration. He is a specialist in the establishment and execution of strategic public-private and public- public collaborations based on the deployment of high value translational research infrastructure for medicine.

Mr. Ussi has been co-responsible for the development of several ongoing public private partnerships and spin-out companies, among which the €130 million VU University Medical Imaging Center Amsterdam, and the Dutch Imaging Hub, a public-private translational initiative with Roche-Genentech and three Dutch university medical centres.

DAY 3: POLICY & FUNDING

Ekaterina Zaharieva

European Commissioner for Startups, Research and Innovation

Ms. Ekaterina Zaharieva is the European Commissioner for Startups, Research and Innovation. She is a Bulgarian lawyer with a long political career. She held various posts including Deputy Prime Minister for Judicial Reform, Deputy Prime Minister for Economic Policy, Minister of Foreign Affairs, Minister of Justice, Minister of Regional Development and Public Works, Head of Cabinet of the President, member of parliament.

She specialized at ENA School of Administration, Paris, at the European Commission for Democracy through Law (Venice Commission) – Council of Europe, and at the Institute of Public Administration, Dublin, Ireland.

Olivér Várhelyi

European Commissioner for Health and Animal Welfare

Mr. Olivér Várhelyi serves as European Commissioner for Health and Animal Welfare since December 2024, in the second von der Leyen Commission, having previously held the position of Commissioner for Neighbourhood and Enlargement in 2019-2024. As Health Commissioner, Várhelyi's mandate focuses on completing the European Health Union by diversifying supply chains, improving access to advanced treatments, and boosting health system competitiveness and resilience. Some of his key priorities include proposing a Critical Medicines Act, concluding the pharmaceutical legislation reform, developing a European Biotech Act, implementing the European Beating Cancer Plan, completing the European Health Data Space and making proposals to scale up genome sequencing capacities.

Mr. Várhelyi is a Hungarian lawyer and diplomat who graduated in law from the University of Szeged and earned a Master of European Legal Studies from Aalborg University in Denmark. His extensive diplomatic career includes serving as Hungary's Permanent Representative to the EU (2015-2019) and Deputy Permanent Representative (2011-2015). Earlier in his career, he served as Head of Unit for Industrial Property Rights in the European Commission's Directorate-General for Internal Market and Services, and held legal and diplomatic roles in the Hungarian Ministry of Foreign Affairs and Ministry of Justice.

Roberta Metsola

President of the European Parliament

Ms. Roberta Metsola was elected President of the European Parliament in January 2022, becoming its youngest-ever leader, and re-elected in July 2024, the first woman to serve two terms. As President, she has driven reforms to make the Parliament more modern, efficient and accountable, while engaging citizens across EU Member States and candidate countries.

First elected to the European Parliament in 2013, Ms. Metsola has since been re-elected in 2014, 2019 and 2024. In 2020, she became the first Maltese Vice-President of the Parliament, responsible for relations with national parliaments and interreligious dialogue. She previously coordinated the EPP Group in the Committee on Civil Liberties and was rapporteur on the FRONTEX regulation.

A lawyer specialized in European law, Ms. Metsola began her career in Malta's EU representation and the office of the High Representative for Foreign Affairs.

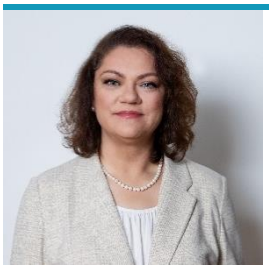
Jennifer Carroll MacNeill

Minister of Health, Ireland

Dr. Jennifer Carroll MacNeill TD is the Minister for Health, appointed by the Taoiseach in January 2025. She previously served as Minister of State for European Affairs & Defence, and before that as Minister of State at the Department of Finance with responsibility for Financial Services, Credit Unions and Insurance.

Re-elected as TD for Dún Laoghaire in 2024, she was first elected to the Dáil in 2020 and has served on the Public Accounts Committee, the Justice Committee and the Committee on the Implementation of the Good Friday Agreement. She was also a member of the British–Irish Parliamentary Assembly.

Before entering politics, Minister Carroll MacNeill qualified as both a solicitor and barrister. She holds a PhD in Public Policy, focusing on political institutions and the judiciary, and has worked as a legal adviser and special adviser in several government departments, including Justice, Housing, and Children and Youth Affairs.



Marija Jakubauskienė

Minister of Health, Lithuania

Ms. Marija Jakubauskienė is a public health expert, researcher and academic leader currently serving as the Minister of Health of the Republic of Lithuania. She brings to the role more than two decades of experience in public health science, biomedical research and health policy development.

Ms. Jakubauskienė earned a PhD in Biomedical Sciences from the Faculty of Medicine at Vilnius University and additionally holds an MBA in Entrepreneurship.

Ms. Jakubauskienė held several senior academic positions - including Associate Professor and Director of the Institute of Health Sciences at Vilnius University's Faculty of Medicine. Her leadership experience also extends to internationally: she served as Vice-Dean for International Affairs and Programmes and played an active role in advancing public mental health through her contributions to the European Public Health Association (EUPHA), where served as President of the Public Mental Health Section and as a member of the International Scientific Committee.

Ms. Jakubauskienė research interests included health policy, population health science, mental health, health system and policy research.

Vytenis Andriukaitis

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

MEP Vytenis Andriukaitis is a Lithuanian politician, medical doctor and Member of the European Parliament. He previously served as Minister for Health of Lithuania (2012–2014) and as European Commissioner for Health and Food Safety (2014–2019). As European Commissioner, one of his major achievements was the establishment of the European Reference Networks, which advanced the care and treatment of patients with rare diseases.

MEP Andriukaitis is a co-author and signatory of the Independence Act of Lithuania (1990) and co-author of the Constitution of the Republic of Lithuania (1992). He was a founder of the Lithuanian Social Democratic Party, later serving as its Chairman (1999–2001) and is currently its Honorary Chairman.

Trained as a cardiovascular surgeon with over 20 years' experience, MEP Andriukaitis also holds a degree in History from Vilnius University. Since 2020, he has been Special Envoy of the WHO for Universal Health Coverage in the European region.

Alexis Arzimanoglou

Coordinator of European Reference Network for all rare and complex epilepsies

Prof. Alexis Arzimanoglou serves as Coordinator of the European Reference Network for Rare and Complex Epilepsies (ERN EpiCare) since 2019 and as Director of the Epilepsy and Neurophysiology Unit at San Juan de Dios Children's Hospital in Barcelona. Between 2008 and 2023, he directed the Epilepsy, Sleep and Paediatric Neurophysiology Department at the University Hospitals of Lyon, France, establishing himself as a leading figure in paediatric epileptology. His clinical and research activities mainly focus on the pharmacological and surgical management and genetics of childhood epilepsies and topics related to cognitive function and dysfunction in children with focal epilepsies.

Prof. Arzimanoglou is an elected member of the European Executive Board of the International League Against Epilepsy (ILAE) as well as a member of its Education Committee and Paediatric Epilepsy Surgery Task Force. In the past he also served as

President of the French Chapter of ILAE and Chair of the Scientific Committee of the European Paediatric Neurology Society (EPNS). His numerous accolades include the ILAE Ambassador for Epilepsy award, the EPNS Aicardi Award for excellence in paediatric neurology (2017), and the European Epilepsy Education Award (2022). He is Editor-in-Chief Emeritus of the ILAE educational journal Epileptic Disorders, has published over 200 peer-reviewed articles and authored/co-authored seven books. Board certified in Neurology in 1989, he trained in neurology and paediatric neurology in London and Paris.

Stine Bosse

Member of the European Parliament (Renew, Denmark)

Ms. Stine Bosse is a Member of the European Parliament (MEP) representing Denmark in the Renew Europe Group. Currently serving as Vice-Chair of the Committee on Public Health (SANT), she has been advocating for a Rare Disease Action Plan at EU level that better connects ERNs and the European Health Data Space and equips primary care doctors across Europe have access to shared data and AI tools.

MEP Bosse brings extensive business leadership experience to European politics. She served as Group CEO of Tryg, the largest Nordic insurer, from 2003-2011, and was identified by the Financial Times in 2009 as "the 22nd most influential businesswoman in the world." Her business career includes board positions with major European companies including Allianz SE, Nordea Bank, and Grundfos. Bosse's commitment to global development and health policy predates her parliamentary career. In 2010, UN Secretary-General Ban Ki-moon appointed her as an Advocate for the Millennium Development Goals. She has served as President of the European Movement in Denmark since 2015 and Chair of BankNordik since 2015.

Silvio Brusaferro

Former President of the Italian Institute of Health (2019-2023)

Prof. Silvio Brusaferro served as President of the Italian National Institute of Health (Istituto Superiore di Sanità) from 2019 to 2023, after initially serving as Commissioner in 2019. During his tenure at the Italian National Institute of Health, he led Italy's public health response during critical periods and represented Italy at international health forums, including the WHO Regional Committee meetings.

Currently, he is Full Professor of Hygiene and Public Health at the University of Udine, where he has worked since 2001, and served as Head of the Department of Medicine from 2016-2019. He also coordinated the European Network of Professionals in Infection Prevention and Control (EUNETIPS). Since 2011 he also heads the Accreditation, Quality and Clinical Risk Management Unit at Friuli Central Healthcare and University Trust (including Udine Academic Hospital), demonstrating his expertise in healthcare quality and safety systems. Prof. Brusaferro obtained his medical degree from the University of

Trieste in 1985 and has built an extensive career in public health administration and academia.

Nicolás González Casares

Member of the European Parliament (S&D, Spain) and member of Panel for the Future of Science and Technology (STOA)

Nicolás González Casares is a nurse and politician with a strong background in public health and emergency medicine. A graduate of the University of Santiago de Compostela, he worked in the 061 Emergency Service and co-authored the "Manual of Advanced Life Support in Pre-Hospital Emergencies" (2007).

His political career began locally, where he served as Deputy Mayor and Councillor for Urbanism, Social Services, Health and Sport in Lalín.

In the European Parliament, he is a member of the Committee on Public Health (SANT) and the Committee on Industry, Research and Energy (ITRE) and Fisheries (PECH). He is also a substitute member in the Committee on the Environment, Climate and Food Safety (ENVI).

Throughout these years, Casares has been able to work directly on many health matters, such as the European vaccination strategy or the strengthening of European mechanisms in the face of health emergencies, beating cancer policy, against the shortage of medicines or in favor of the affordability and availability of medicines.

MEP González Casares has been the Parliament's rapporteur for a regulation strengthening the European Medicines Agency's role in crisis preparedness, the Socialist shadow rapporteur for the report of the Special Committee on Beating Cancer and the Pharmaceutical Package Directive. He has been appointed recently as rapporteur for the INL on the European Union Rare Disease Action Plan.

Giacomo Chiesi

Executive Vice President, Global Rare Diseases, Chiesi Group

A lifelong advocate for patients, Mr. Giacomo Chiesi serves as Executive Vice President of Chiesi Global Rare Diseases, where he leads efforts to develop and deliver transformative therapies for individuals living with rare and ultra-rare conditions. With a mission to listen deeply, act boldly, and alleviate suffering, Giacomo is focused on building an organization that centers the voices of patients at every stage of innovation.

Previously, he led Global Corporate Development for the Chiesi Group, where his strategic vision helped expand Chiesi into new markets across North America, Europe, and Asia and laid the foundation for the launch of the rare disease business unit.

He is also Managing Partner of Chiesi Ventures, a joint venture with Pappas Capital, focused on starting new rare disease companies. Giacomo serves on the Chiesi Group Board of Directors and on the Advisory Board of the Orphan Therapeutics Accelerator, a

non-profit biotech organization focused on completing development and commercializing therapies for ultra-rare conditions. A Champion of Hope awardee and PharmaVoice 100 Honoree, Giacomo remains committed to shaping a future where all patients feel heard, seen, and supported.

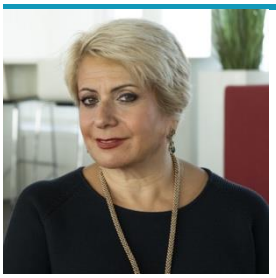
Avril Daly

President, EURORDIS

Ms. Avril Daly has been President of EURORDIS since November 2022. She was first elected to the EURORDIS Board of Directors in 2009 and served as Vice-President from 2012 to 2022. Ms. Daly is also CEO of Retina International, the global umbrella organisation promoting research into retinal diseases such as Retinitis Pigmentosa, Usher Syndrome and Macular Degeneration, representing patients in 50 countries and driving policy advocacy, scientific collaboration, and capacity-building.

Previously, Ms. Daly was CEO of Fighting Blindness Ireland, where she led strategic development and raised awareness of retinal degenerative diseases nationally. She is Chairperson of Rare Diseases Ireland and has played a key role in shaping Ireland's National Plan for Rare Diseases.

She represents Retina International at the European Patients' Forum and EURORDIS at international conferences. A Business & Media Studies graduate, she was diagnosed with Retinitis Pigmentosa in 1998, which continues to inspire her leadership in the rare disease community.



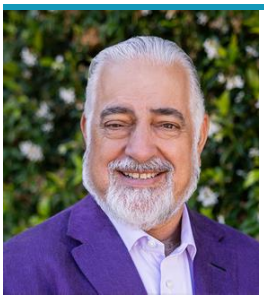
Joanna Drake

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

Ms. Joanna Drake is the Deputy Director-General for Planet, People and Science for Policy in the European Commission's Directorate-General for Research and Innovation (DG RTD) since 2021. She currently serves as the EU Mission on Cancer and Acting Director for the Clean Planet Directorate, focusing on science for policy, environment, climate, energy, culture, and health.

For the last 40 years, she has contributed to the EU in several roles at the national and European level. Between 2016 and 2021, she was Deputy Director-General in DG Environment where she chaired a crosscutting Task Force spear-heading strategic positions for the DG on (inter-alia) the post-2020 Commission financial framework negotiations, Brexit co-ordination, the urban agenda and the future-proofing of the EU's environmental acquis. Between 2010 and 2015 she was director for SME's and Entrepreneurship in the DG for Internal Market, Industry, Entrepreneurship and SMEs (DG GROW). During her tenure in DG GROW she also led the Commission's Task Force on The Collaborative Economy, New Business Models and SME's.

Before joining the Commission, she lectured full-time in European and Comparative Law at the University of Malta and held various legal and management positions in the private sector. She holds a doctorate in laws from the University of Malta and a post-graduate degree in Advanced European Legal Studies from the College of Europe in Bruges.



Emil Kakkis

CEO Ultragenyx

Dr. Emil Kakkis is the Chief Executive Officer, President and Founder of Ultragenyx Pharmaceutical, established in 2010 to accelerate the development of therapies for rare and ultra-rare genetic diseases. He pioneered a patient-centered development model that transformed clinical trial design, endpoints and access strategies for rare disease treatments.

Before founding Ultragenyx, Dr. Kakkis worked at Harbor-UCLA Medical Center, where he developed enzyme replacement therapy for MPS I, and later at BioMarin, where he led the development and approval of treatments for MPS I, MPS VI and PKU, among others.

Beyond his industry achievements, he founded the EveryLife Foundation for Rare Diseases to advance policy and innovation in rare disease research. His contributions have been recognized with multiple awards, including BIO's Henri Termeer Visionary Leadership Award. Dr. Kakkis holds an M.D. and Ph.D. from UCLA, and began his career as a pediatrician and medical geneticist.

András Tivadar Kulja

Member of the European Parliament (EPP, Hungary)

MEP András Tivadar Kulja is a Hungarian surgeon, digital health expert and politician of the Tisza Party, elected to the European Parliament in 2024. He serves as Vice-Chair of the Committee on Environment, Public Health and Food Safety (ENVI) and Deputy Coordinator for the European People's Party in the Committee on Public Health (SANT).

MEP Kulja studied medicine at Semmelweis University, where he also earned a master's degree in healthcare administration and management and later lectured on health and science communication. He expanded his expertise in e-health and workforce development at IESE Business School. At Semmelweis he co-founded courses on health communication.

During the Covid-19 pandemic, MEP Kulja gained prominence for his health education videos on social media, particularly TikTok, reaching more than 100 million views. His channel, with over 370,000 followers, continues to provide accessible health information and insights on healthcare innovation and medical technology.

Peter Liese

Member of the European Parliament (EPP, Germany)

Dr. Peter Liese has served as a Member of the European Parliament (MEP) since 1994, representing Germany in the European People's Party (EPP) Group. He is a qualified physician who graduated from the Institute of Human Genetics at the University of Bonn in 1993 and, before being elected as an MEP, practiced medicine in development projects in Central America (1992-1994), followed by work in a general practice until 2002. His medical background uniquely positions him to address health policy issues in the European Parliament, where he serves as EPP Coordinator in the Committee on the Environment, Public Health and Food Safety (ENVI) since 2009 and acts as the EPP spokesperson for health and environment policy.

Since 2020, MEP Liese has served on the Special Committee on Beating Cancer, applying his medical expertise to cancer policy development. His political engagement extends beyond the European level, having served on the CDU federal executive from 2012-2018 and participating in coalition negotiations in Germany. He has also chaired the EPP Health Ministers Meeting alongside Commissioner Stella Kyriakides, gathering center-right health ministers ahead of Employment, Social Policy, Health and Consumer Affairs Council meetings.

Alexander Natz

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

Dr. Alexander Natz serves as Secretary General of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE) in Brussels, representing innovative pharmaceutical and biotech companies towards the EU institutions, EMA and the relevant EU stakeholders and associations. He combines his EUCOPE leadership with his position as Partner at Novacos Rechtsanwälte, where he advises innovative life sciences companies, including start-ups of the pharma and biotech sector, on European and German life sciences law covering pharmaceuticals, medical devices, food and cosmetics.

His legal expertise encompasses EU and German law perspectives on pharmaceutical regulation, having previously worked as Head of the Brussels Office of Bundesverband der Pharmazeutischen Industrie (BPI) from 2008-2013. Before, he worked as a lawyer at Sträter Law Firm in Germany with a special focus on managed entry agreements and licensing of pharmaceuticals. His career also included experience in competition law with the European Commission and pharmaceutical industry work. He conducted research on international pharmaceutical law as a research assistant at Duke University and earned his doctorate under supervision of former European Court of Justice judge Prof. Dr. Ulrich Everling.

Leonidas A. Phylactou

CEO and Medical Director of The Cyprus Institute of Neurology and Genetics

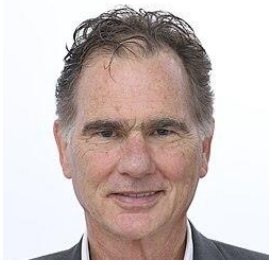
Prof. Leonidas Phylactou is the Chief Executive Officer and Medical Director of the Cyprus Institute of Neurology and Genetics (CING) and the Provost of the Cyprus School of Molecular Medicine, the postgraduate school of the CING.

He received his Bachelor's degree in Medical Biochemistry with honours from the University of Birmingham, UK in 1992 and his PhD in Molecular Genetics/Gene Therapy from the same University in 1995 in collaboration with the University of Connecticut Health Center, USA. Upon completion of his studies, he moved to Oxford University as a post-doctoral scientist where he led a research team on the use of catalytic RNAs as tools for gene therapy in the brain and muscle. Upon returning to Cyprus in 1998 he was employed at The Cyprus Institute of Neurology and Genetics (CING) where he established a research team to investigate and develop novel ways to study gene function and exploit gene therapy approaches. In 2005, he was appointed as the Head of the Department of Molecular Genetics, Function and Therapy, which he still directs.

From 2012 until 2015, Prof. Phylactou served as the first Dean of the newly established post-graduate school of the CING, the School of The Cyprus Institute of Neurology and Genetics.

From March 2014 to July 2015, Prof. Phylactou served as the Acting CEO and Medical Director of the CING and Acting Provost of the School of the CING. In November 2015, he was appointed as the CEO and Medical Director of the Cyprus Institute of Neurology and Genetics and the Provost of the School.

During his scientific career Prof. Phylactou has secured research funding from several local and international funding organizations and published extensively in the areas of molecular genetics, gene function and gene therapy. He also serves on the Editorial Boards of international peer-reviewed journals. Prof. Phylactou received the 'Distinguished Researcher Award 2023' and the 'Cyprus Quality Leader of the Year 2024'.



Nikos Papandreou

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

Mr. Nikos Papandreou is a Greek politician, businessman, cultural commentator and writer as well as a Member of the European Parliament (MEP) representing Greece in the Group of the Progressive Alliance of Socialists and Democrats (S&D) since May 2023. As a member of the Committee on Public Health (SANT), and building on his diverse background spanning politics, economics, and healthcare policy, MEP Papandreou focuses particularly on cancer policy reform. He advocates for stronger policies to reduce cancer care disparities, promote innovation, and enhance collaboration among EU countries, research organisations, and patient advocacy groups.

MEP Papandreou holds degrees in economics from Princeton University, including studies at the Woodrow Wilson School of Public and International Affairs, and Yale University. Before entering European politics, Papandreou worked with international institutions including the World Bank and served in advisory roles within Greek socialist governments, having witnessed Greece build a national health system from the ground up.

Vlad Voiculescu

Member of the European Parliament (Renew, Romania)

Mr. Vlad Vasile Voiculescu serves as a Member of the European Parliament (MEP) for Romania in the Renew Europe Group since July 2024. He currently chairs the European Parliament's Intergroup on Cancer & Rare Diseases and serves as coordinator for Renew Europe at the EP's Committee on Public Health (SANT).

MEP Voiculescu has extensive experience in healthcare leadership, having served twice as Romania's Minister of Health (May 2016-January 2017 and December 2020-April 2021). His background uniquely combines healthcare policy expertise with patient advocacy, particularly in oncology. Between 2008 and 2016, he founded and led (as a volunteer) the Cancer Drugs Volunteer Network, helping over 2,000 Romanian cancer patients access essential medicines through a network of 400+ volunteers. Between 2013 and 2016 he served as Vice President (volunteer) of the European Cancer Patient Coalition (ECPC), and between 2019 and 2021 he was elected chair of the Patient Advocacy Working Group at the European Society for Medical Oncology (ESMO). He holds advanced degrees in finance and has demonstrated consistent commitment to improving patient access to innovative treatments across Europe.

Alexandra Heumber Perry

CEO, Rare Diseases International

Alexandra Heumber Perry is the Chief Executive Officer of Rare Diseases International, the global alliance of Persons Living with a Rare Disease.

She has dedicated her entire career to contributing to improving healthcare policies to benefit people living with diseases, with a particular focus on neglected and vulnerable people. She has over 20 years of experience in global health with demonstrated capabilities in patient advocacy and multi-stakeholder partnership.

As CEO of RDI, Ms. Heumber Perry sets the strategic direction, drives the organization's goals of raising awareness of rare diseases, improving access to diagnostics, treatment and care, advocating for the rights of patients around the world and representing its members and enhancing their capacities to ultimately improve lives of persons living with a rare disease.

Johan Prevot

Executive Director, International Patient Organisation for Primary Immunodeficiencies (IPOPI)

Mr. Johan Prevot is a healthcare professional with over 22 years of experience in patient advocacy and health policy. He currently serves as the Executive Director of the International Patient Organisation for Primary Immunodeficiencies (IPOPI), where he leads global activities and advocacy campaigns.

Mr. Prevot is actively involved in various healthcare organizations, working to improve patient access to early diagnosis and treatment for rare diseases. He has been a strong advocate for equitable access to plasma therapies and played a key role in launching the International Neonatal Screening Day. He is committed to collaborating with stakeholders to achieve common objectives and priorities in healthcare.

Maurizio Scarpa

Director Regional Coordinating Centre for Rare Diseases, University Hospital Udine, Coordinator European Reference Network For Hereditary Metabolic Diseases (MetabERN) and Founder of Brains for Brain Foundation (Co-Chair)

Maurizio Scarpa, MD PhD, paediatrician, is the Director of the Regional Coordinating Centre for Rare Diseases at the University Hospital of Udine, Italy. He is Professor of Paediatrics at the Dept. for the Woman and Child Health, University of Padova, Italy, and the Co-Founder of the Brains For Brain Foundation, together with Prof. David Begley, Kings College of London, London, UK. Prof. Scarpa has extensive expertise as a basic scientist in genetics and biotechnology, as well as a clinician in the diagnosis and treatment of paediatric rare disorders; neurometabolic diseases in particular. Together with dr. Christina Lampe he founded the Center for Rare Diseases at the Helios Dr. Horst Schmidt Kliniken in Wiesbaden, Germany. He is especially interested in developing innovative health approaches for the diagnosis and the treatment of metabolic inherited diseases; to this aim he is also collaborating with major biotech companies as an external independent expert. Prof. Scarpa is the Coordinator of the European Reference Network for Hereditary Metabolic Diseases, MetabERN, formed by 101 healthcare providers in 27 EU countries (www.metab.ern-net.eu) to facilitate patient-centered holistic activities to implement knowledge, diagnosis, management and treatment for inherited metabolic diseases.

Birutė Tumienė

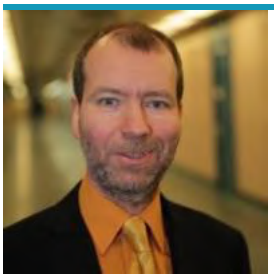
Co-Chair of ERN Board of Member States

Assoc. Prof. Birutė Tumienė serves as co-chair of the ERN Board of Member States since 2023 and has recently expressed her interest to continue this role for the next term. She outlined her priorities, which include managing ongoing actions such as changes to the ERN system, completing the 5-year evaluation, expanding disease coverage, clarifying status of consortia, updating specific criteria and integrating the ERNs into national health

systems. Currently, she heads the Unit for Clinical Genetics and Genomics and the Coordination Center for Rare Diseases at Vilnius University Hospital while also serving as Associate Professor at Vilnius University Faculty of Medicine, where she lectures on genetics and rare diseases.

Assoc. Prof. Tumienė's extensive international engagement spans nearly 20 years in rare diseases activities. In the Joint Action on ERN integration into national systems JARDIN, she co-leads 2 different work packages on rare disease care pathways and ERN sustainability, including revision of National RD Plans and Strategies in the EU. She is also a co-leader of a Workstream for education and capacity building in the European Rare Disease Research Alliance (ERDERA), a member of the IRDiRC Diagnostic Scientific Committee, a National Coordinator of Orphanet Lithuania, and a member of a Panel of Experts in the WHO Collaborative Global Network for Rare Diseases. In 2021, she received the EURORDIS Black Pearl European Leadership Award for her contributions to developing European Reference Networks.

She graduated from Vilnius University Faculty of Medicine, where she completed her residency training in clinical genetics and accomplished PhD studies in rare genetic epilepsies.



Till Voigtländer

Coordinator of Joint Action on Integration of ERNs into National Healthcare Systems (JARDIN)

Dr. Till Voigtländer is associate professor of neurobiology and neurosciences at the Department of Neurology, Division of Neuropathology and Neurochemistry, Medical University of Vienna.

After studying medicine in Heidelberg, Germany, he received his professional training in molecular biology, neuropathology, neurochemistry and neuroimmunology at different universities and institutions in Heidelberg, Berlin, Zurich, and Vienna. Since his board certification as specialist in neurobiology in 2006, he leads a specialised clinical laboratory focussing on the diagnosis of selected rare neurometabolic, neuroimmunological and neurodegenerative diseases.

In Austria, Dr. Voigtländer is country coordinator of Orphanet since 2004, was head of the National Coordination Centre for Rare Diseases at the Austrian Healthcare Institute (2011-2018) and is now the director of the national office for rare diseases (since 2019). He was one of the key participants in the elaboration of the national plan of action for rare diseases (2015) and is currently actively involved in its ongoing implementation.

At the European level, Dr. Voigtländer was part of several former European expert groups and committees and has served three times as co-chair of the Board of Member States for ERNs. Most recently, he was appointed by all Member States as coordinator of the Joint Action on Integration of ERNs into National Healthcare Systems (JARDIN Joint Action), a pioneering three-year EU-funded project aimed at integrating the 24 European

Reference Networks (ERNs) into national healthcare systems across Europe. The project addresses critical aspects of rare disease care, including patient pathways, national reference networks, and data management solutions for rare diseases.

As a result of his extensive contributions to the field of rare diseases, in 2019, Dr. Voigtländer was presented with the European Rare Disease Leadership Award at the EURORDIS Black Pearl Awards.

Tiemo Wölken

Member of the European Parliament (S&D, Germany) and S&D Coordinator for the Environment, Climate and Food Safety

Tiemo Wölken is a German lawyer and politician who has served as a Member of the European Parliament (MEP) since 2016, representing the Social Democratic Party (SPD) within the Progressive Alliance of Socialists and Democrats (S&D). A graduate in European public law from the University of Osnabrück and with a Master's in International Law from the University of Hull, he has been an active voice on health, climate, and digital policy.

In the European Parliament, he is the S&D Coordinator in the Committee on the Environment, Public Health and Food Safety (ENVI) and member of the Committee on Public Health (SANT), as well as a substitute Member of the legal affairs Committee (JURI). MEP Wölken has served as rapporteur on the Health Technology Assessment (HTA) and the pharmaceutical regulation, advancing EU-level reforms for innovation and equitable access to medicines, whilst also working on pharmaceutical patents (SPCs) within the legal affairs Committee. He co-chairs the MEP Interest Group on Antimicrobial Resistance (AMR) and works to strengthen evidence-based and sustainable European health policy.

FUTURE RELEVANT MEETINGS

EU Conference "Advancement of Treatments for Rare Diseases" (5-6 March, Nicosia)

The Cyprus Institute of Neurology & Genetics will host the EU funded Conference "Advancement of Treatments for Rare Diseases" on 5–6 March 2026 in Nicosia, Cyprus, as a flagship event of the Cyprus Presidency of the Council of the European Union 2026.

Funded by Horizon Europe and co-organized with the Cyprus Deputy Ministry of Research, Innovation and Digital Policy, the conference envisages to gather over 30 distinguished speakers and 250 participants from across Europe and beyond, including representatives from EU institutions, healthcare authorities, academia, patient organizations, and industry.

The two-day high-level event aims to strengthen collaboration, foster innovation, and advance research and development of treatments for rare diseases (RDs), aligning with the EU vision to make Europe "the global beacon for life sciences by 2030".

Key objectives include:

- Enhancing collaboration among Member States, healthcare providers, researchers and industry
- Promoting innovation and translational research for new therapies
- Improving regulatory framework for orphan drugs
- Ensuring equitable access to RD treatments across Europe
- Increasing awareness and data-sharing capabilities through secure, AI-enabled infrastructures.

By spotlighting existing EU initiatives, the conference will serve as a strategic platform for policy dialogue and scientific collaboration, reinforcing Europe's leadership in rare disease research while underscoring Cyprus's expanding role in shaping international health policy.

The Conference will be held at the Cyprus Institute of Neurology & Genetics, in Nicosia, the capital of Cyprus — a city rich in history, culture, and scientific collaboration. As part of Cyprus's contribution during its Presidency of the Council of the EU, the Conference offers not only scientific excellence but also a unique setting for networking, exchange, and cooperation.

13th European Conference on Rare Diseases & Orphan Products (ECRD 2026)

Convened by EURORDIS-Rare Diseases Europe and Orphanet

The next ECRD will be held on 3 & 4 June 2026 in Prague and will be titled "Rare Diseases in a Changing & Competitive Europe: Shaping policies to address the unmet needs of people living with rare diseases".

By bringing together people living with rare diseases and patient advocates alongside policymakers, healthcare industry representatives, clinicians, regulators, and Member State officials, EURORDIS leverages the strength of this unique network to advance goal-driven policies that improve the lives of people living with a rare disease and their families.

ECRD 2026 is expected to unite over 500 participants in Prague, with an additional 300+ joining online. Thanks to its hybrid format, the conference will provide an unparalleled platform for connection and collaboration, enabling participants across the rare disease community to exchange knowledge, share experiences, and build partnerships.

ECRD 2026 will serve as a pivotal milestone in the multi-stakeholder inclusive process towards shaping a European Blueprint for Rare Diseases.

The Blueprint will stand as the culmination of this collaborative process, fostering collective ownership and a lasting impact across Europe, while strengthening Europe's contribution to the WHO Global Action Plan on Rare Diseases.

The event will delve into the most pressing discussions on the future of Europe, exploring the multifaceted challenges and opportunities for the rare disease ecosystem. The programme will cover a broad range of policy topics, including therapy development and access to treatments, timely and accurate diagnosis, advances in holistic care, specialised healthcare, health technology assessments, and mental health.