European Reference Network

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

BRAINS FOR BRAIN

HLM RARE

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

01 ABOUT THE INITIATIVE

WHAT INSPIRED US

FOCUS ON:

The importance of research and innovation in driving the Single Market's future success. A core proposal is the **introduction of a "fifth freedom" - the freedom of movement of knowledge**, complementing the existing four freedoms (movement of goods, services, capital, and people)

FOCUS ON:

A roadmap for strengthening Europe's R&I ecosystem, ensuring that Europe remains at the forefront of scientific and technological progress to address pressing societal challenges and drive sustainable economic growth. It calls for a mission-oriented approach to research and innovation, focusing on developing solutions to improve the lives of European citizens

FOCUS ON:

A vision for a more dynamic, innovative, and sustainable European economy.

It calls for establishing a **Research and Innovation Union**, fostering public and private collaboration and **investing in cutting-edge technologies and world-class innovation hubs in life sciences** for advanced therapy medicinal products (ATMPs) and scaling niche know-how in rare diseases.

OUR GOAL: CURE

C	U	R	E
Creating a pan-European space for research and innovation, fully harnessing the 5th freedom.	**Uniting** the rare diseases community under the Draghi, Letta, Heitor and Niinistö reports	**Reinforcing** the ecosystem for innovation and care of rare and complex diseases.	**Enhancing** the European Reference Networks Ecosystem to drive scientific, medical and industrial excellence.

European Reference Network

Share. Care. Cure.

02 ABOUT THE DECLARATION

WHAT IT IS & OBJECTIVES

Persistent fragmentation across legislation, funding and implementation continues to limit equitable access and innovation in rare diseases. The Declaration on a European Innovation and Care Ecosystem for Rare and Complex Diseases responds by setting out a coherent, pan-European innovation and care ecosystem, with the European Reference Networks as a central pillar.

What it is	The objective	The short-term goal	The medium-to-long term goal
A political and strategic commitment to fundamentally transform the European ecosystem for rare and complex diseases. **A bold shared vision** for better alignment between research, innovation, health systems, patients and industry.	To **define clear strategic priorities** to set the direction for future action and mobilize strong political support from EU institutions, relevant agencies, national authorities, and the wider stakeholder community.	**The establishment of Working Groups,** tasked with developing concrete and measurable sub-Action Plan to achieve these ambitions.	**Accelerate progress for rare and complex diseases**, notably by increasing the number of available treatments and by universalizing newborn screening and advanced diagnostics.

WHO THE DECLARATION REPRESENTS

The Declaration reflects a broad, multi-stakeholder coalition engaged in advancing rare and complex disease research, innovation and care across Europe. This diversity underpins the Declaration's legitimacy and supports a shared, system-level approach to addressing fragmentation and unmet needs.

It represents:

Patients & patients organisations

Healthcare professionals

European Reference Network

European Reference Networks

Academia & researchers

Industry

03 A ROADMAP FOR ACTION

THE 8 PRIORITY ACTIONS

8 Recommendations

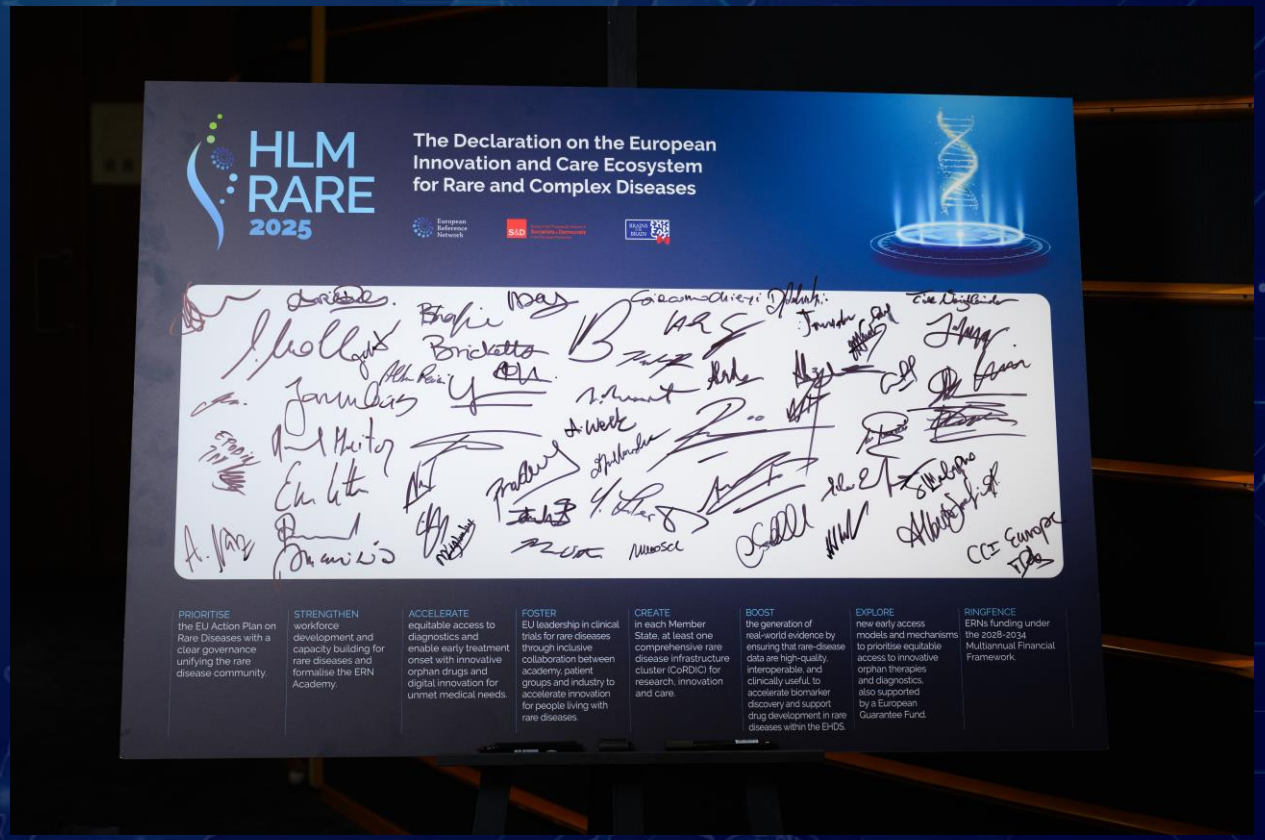
- Prioritise the EU Action Plan on Rare Diseases with a Clear Governance unifying the Rare Disease Community
- Strengthen Workforce Development and Capacity Building for Rare Diseases and formalise the ERN Academy
- Accelerate Equitable Access to Diagnostics and enable Early Treatment Onset with Innovative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

04 FROM DECLARATION TO ACTION

SIGNATORIES OF THE DECLARATION

More than 50 organisations have signed the Declaration during the event, including:

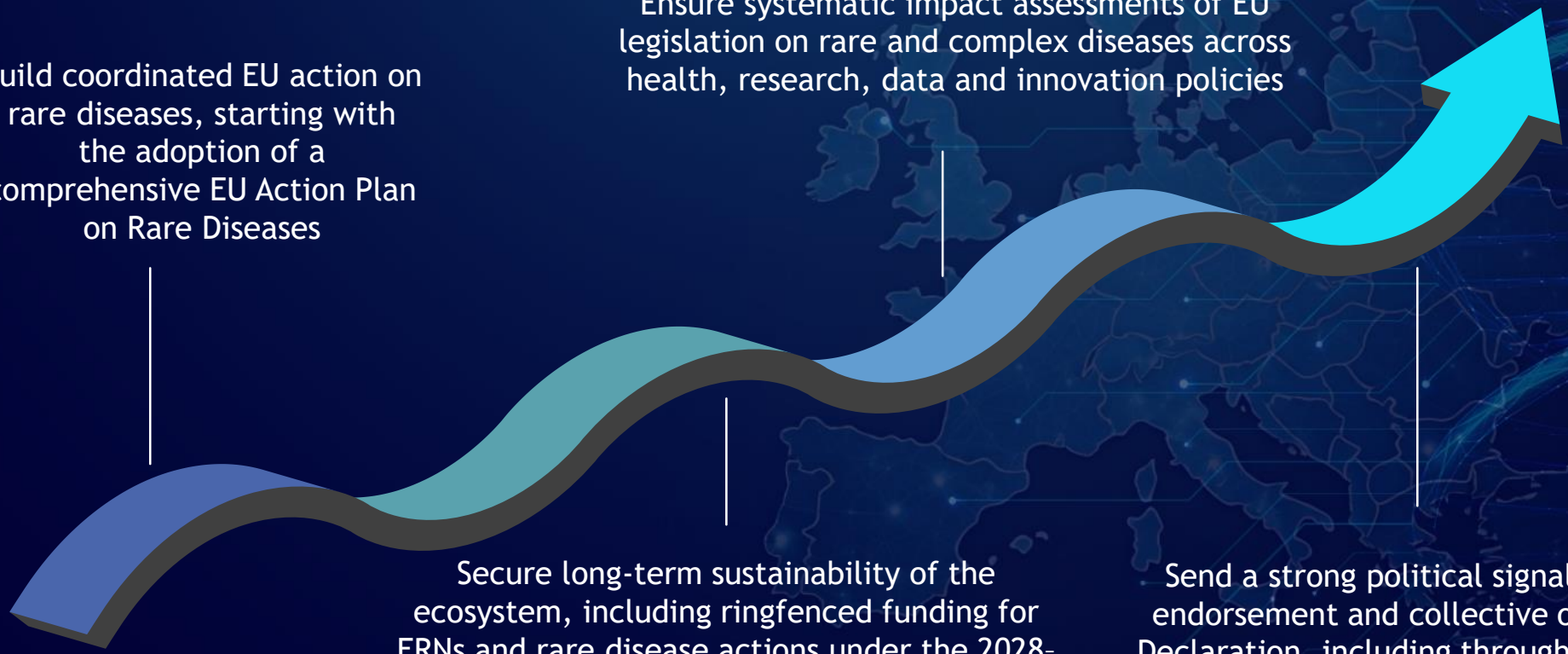
- The 2 co-hosts
- 14 ERN coordinators
- 9 Members of the Parliament
- 2 former policy-makers
- 6 patient organisations
- 10 professional societies & research institutes/organisations/projects
- 8 industry representatives



The Declaration remains open for signature at this link.

WHAT HAPPENS NEXT WITH THE DECLARATION

The Declaration is a vehicle to mobilise political commitment and enable coordinated, measurable EU-level action on rare and complex diseases. Therefore, we are now garnering community support, including through a growing number of official endorsing organisations as well as individuals.



Build coordinated EU action on rare diseases, starting with the adoption of a comprehensive EU Action Plan on Rare Diseases

Ensure systematic impact assessments of EU legislation on rare and complex diseases across health, research, data and innovation policies

Secure long-term sustainability of the ecosystem, including ringfenced funding for ERNs and rare disease actions under the 2028-2034 MFF

Send a strong political signal by broadening endorsement and collective ownership of the Declaration, including through the launch of an EU Citizen Initiative by the end of 2026 to collect 1M signatures

HOW THE DECLARATION CAN BE USED

As a reference for policy positions
To support coherent positions across EU institutions, Member States and stakeholders, aligned with existing EU strategies and legislative frameworks.

As an advocacy tool at national level
To support dialogue with national authorities, promote alignment with EU priorities, and encourage integration of ERNs into national systems.

As a framework for future initiatives and funding
To inform the design of follow-up actions, pilot projects and investment priorities under EU and national programmes, including the next Multiannual Financial Framework.

Using rare diseases as a blueprint for EU health innovation
To demonstrate how integrated approaches to research, data, innovation and care can be designed and scaled across other health areas.

HLM4RARE 2026: A STRATEGIC FORUM FOR DELIVERY

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

Position HLM4RARE as a permanent strategic forum bringing together EU institutions, Member States, ERNs, patients, academia and industry

Support coordination, monitoring and continuity, linking policy commitments with implementation timelines and funding cycles

Convene multi-stakeholder Working Groups to develop and advance sub-action plans aligned with the Declaration's priority recommendations

Launch detailed follow-up sub-action plans at HLM4RARE 2026, ensuring accountability and momentum beyond the Declaration

THANK YOU!

ANNEX: A DEEP DIVE INTO THE DECLARATION RECOMMENDATIONS

PRIORITY ACTION 1: EU ACTION PLAN FOR RARE DISEASES

THE CHALLENGE

- Rare disease policy remains fragmented across initiatives, legislations and funding streams, limiting coherence and impact.
- The absence of a single governance mechanism hinders alignment between EU institutions, Member States, ERNs and stakeholders.
- There is no shared framework to monitor implementation and results across rare disease policies at EU level.

WHAT THE DECLARATION CALLS FOR

- Adoption of a comprehensive EU Action Plan on Rare Diseases, anchored in a cross-cutting Rare Disease Mission.
- Establishment of a high-level Consultative Group to assess coherence, reduce regulatory overlaps and optimise cross-border healthcare frameworks.
- Development of a shared dashboard with clear indicators, targets and metrics to track progress and outcomes.

PRIORITY ACTION 2: WORKFORCE DEVELOPMENT

THE CHALLENGE

- Rare disease expertise remains insufficiently embedded in health education and training, contributing to delayed diagnosis and uneven care.
- Workforce development and knowledge exchange are fragmented and under-resourced, with limited structured EU-level coordination.
- Gaps in digital and AI-related skills limit the effective use of innovation and cross-border collaboration within ERNs.

WHAT THE DECLARATION CALLS FOR

- Formalise the ERN Academy as the EU framework for training, skills development and knowledge exchange in rare diseases.
- Embed rare disease education in medical and nursing curricula, aligned with the “Choose Europe” initiative and the Fifth Freedom.
- Strengthen EU and national investment in workforce capacity, including digital and AI-enabled skills, and support training for patients and families.

PRIORITY ACTION 3: EQUITABLE ACCESS TO DIAGNOSTICS AND EARLY TREATMENT

THE CHALLENGE

- Access to innovative orphan therapies and diagnostics remains uneven across Member States, particularly affecting smaller countries with limited fiscal capacity.
- Existing early access, procurement and reimbursement pathways are fragmented and poorly coordinated, delaying patient access.
- Structural inequities limit the ability of national systems to absorb and sustain innovation, despite EU-level approvals.

WHAT THE DECLARATION CALLS FOR

- Develop financially sustainable early access models for orphan therapies and diagnostics, based on transparent, value-based and patient-centred criteria.
- Strengthen pan-European pathways and cross-country collaboration with industry on early access, procurement and reimbursement mechanisms.
- Pilot a European Guarantee Fund, on a small and targeted scale, to address structural inequities and assess its feasibility in improving timely access across the EU.

PRIORITY ACTION 4: FOSTER EU LEADERSHIP IN CLINICAL TRIALS FOR RARE DISEASES

THE CHALLENGE

- Europe's share of global rare-disease clinical trials is declining, with development increasingly shifting to the US and Asia.
- Lengthy and fragmented approval processes, particularly for multi-country trials, delay patient access and deter investment.
- Limited coordination between academia, patient groups, industry, ERNs and regulators slows the translation of trial results into clinical practice.

WHAT THE DECLARATION CALLS FOR

- Strengthen inclusive public-private collaboration between academia, patient organisations and industry to accelerate rare-disease clinical research.
- Reduce timelines and administrative barriers through a risk-based, fast-track regulatory approach for rare-disease clinical trials.
- Support EU-level funding and coordination mechanisms that reward partnerships, harmonise ethical review processes and facilitate rapid uptake of validated therapies across Member States.

PRIORITY ACTION 5: COMPREHENSIVE RARE DISEASE INFRASTRUCTURE CLUSTERS (CORDIC)

THE CHALLENGE

- Research, innovation and care for rare diseases remain dispersed across institutions, limiting efficiency, visibility and patient navigation.
- Not all Member States have sufficient infrastructure to support advanced diagnostics, translational research and clinical trials.
- Interoperability between biobanks, registries, genomic initiatives and centres of expertise remains incomplete, constraining pan-European collaboration.

WHAT THE DECLARATION CALLS FOR

- Establish at least one Comprehensive Rare-Disease Infrastructure Cluster (CoRDIC) in each Member State, integrating research, innovation and care capacities.
- Ensure CoRDICs function as identifiable access points for patients, supporting early diagnosis, clinical trials and state-of-the-art care.
- Strengthen interconnections and interoperability between national infrastructures and EU-level initiatives, with sustained and predictable funding to support a pan-European ecosystem.

PRIORITY ACTION 6: BOOST REAL-WORLD EVIDENCE GENERATION

THE CHALLENGE

- Rare-disease data are fragmented, heterogeneous and uneven in quality, limiting their clinical and regulatory usefulness.
- Limited interoperability between registries, biobanks and EHRs constrains cross-border research, care coordination and evidence generation.
- The absence of shared standards and analytics frameworks slows the responsible uptake of AI-enabled innovation.

WHAT THE DECLARATION CALLS FOR

- Coordinate end-to-end rare-disease data collection and governance across ERN registries, biobanks and EHRs, aligned with FAIR principles and the EHDS.
- Improve data quality and clinical utility through harmonised standards, shared validation metrics and integration of real-world data and PROMs.
- Enable federated analytics and common data models to support privacy-preserving cross-border collaboration, AI/ML development and regulatory-grade evidence.

PRIORITY ACTION 7: NEW BUSINESS MODELS AND MECHANISMS FOR EQUITABLE ACCESS

THE CHALLENGE

- Access to early and accurate diagnosis remains uneven across Member States, with significant delays for many people living with rare and complex diseases.
- Newborn screening programmes, digital tools and advanced diagnostics are implemented inconsistently, limiting their impact and scalability.
- Fragmented data collection and evaluation frameworks slow the responsible uptake of digital and AI-enabled solutions across care pathways.

WHAT THE DECLARATION CALLS FOR

- Accelerate equitable access to early diagnosis, including through the advancement of evidence-based newborn screening programmes across the EU.
- Support coordinated EU-level action to align rare disease data collection and enable the harmonised assessment and responsible use of digital and AI-enabled applications.
- Strengthen integrated care pathways, ensuring that early diagnosis translates into timely treatment onset and improved outcomes across Member States.

PRIORITY ACTION 8: RINGFENCE ERNS FUNDING UNDER MFF

THE CHALLENGE

- Funding for the ERNs and rare disease actions remains fragmented, short-term and project-based, limiting sustainability and strategic planning.
- Insufficient coordination between EU and national funding streams creates gaps and discontinuities in ERN operations and impact.
- Complex administrative procedures and overlapping governance reduce efficiency and long-term investment capacity.

WHAT THE DECLARATION CALLS FOR

- Ringfence ERN and rare disease funding within the 2028–2034 Multiannual Financial Framework.
- Ensure continuity across EU and national programmes, linking successors of Horizon Europe and EU4Health.
- Move from project-based funding to durable mechanisms, including multi-country consortia and pooled investments.
- Simplify governance and administrative procedures to support long-term planning and reduce duplication.