HLM RARE 2025

European Reference Networks

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

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The Declaration on the European Innovation and Care Ecosystem for Rare and Complex Diseases

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INTRODUCTION

Introduction

The Declaration on the EU Innovation and Care Ecosystem for Rare and Complex Diseases was developed in response to the reports by M. Draghi, M. Heitor, E. Letta, and S. Niinistö, the statements issued by the European Commission, and the active engagement of industry and stakeholders, ultimately reflecting the pressing necessity of addressing growing unmet needs.

It is the result of an initiative convened by Member of the European Parliament Vytenis Andriukaitis and Maurizio Scarpa MD PhD, Coordinator of the MetabERN, European Reference Network for Inherited Metabolic Diseases, founder of the Brains for Brain Foundation, in collaboration with ERN Coordinators and the Brains for Brain Foundation, supported by a grant from the European Union[1].

This collaboration, which gathers multistakeholder support[2], aims to address the persistent fragmentation in rare disease diagnosis, care, and research across Europe, while also addressing the urgent needs of more than 30-36 million patients in the EU. We are united by the commitment to foster scientific excellence, strengthen cross-border collaboration, address unmet needs, improve the lives of people living with rare diseases, and enhance industrial competitiveness, thereby establishing a cohesive and world-leading ecosystem.

The Declaration examines how to strategically place research and innovation at the heart of EU life science and health policy, using rare diseases as a model to showcase the EU's added value.

1 HaDEA Grant Agreement No 101233680

2 We extend our gratitude to MEP Vytenis Andriukaitis and Prof. Maurizio Scarpa, the dedicated ERN Coordinators: Alexis Arzimanoglou (EpiCare), Holm Graessner (ERN-RND), Luca Sangiorgi (ERN-BOND), María del Mar Mañú Pereira (EuroBloodNet), and Ruth Ladenstein (ERN-PaedCan). We further acknowledge and thank the esteemed members of the HLM RARE Steering Committee, the sponsors of the High-Level meeting, and all other contributors whose expertise and efforts were instrumental in the successful drafting and review of the Declaration on the European Innovation and Care Ecosystem for Rare and Complex Diseases.

THE PREAMBLE

1. Main reports call for united actions, building viable ecosystems

The EU is at a critical moment. If we want it to remain a global leader in science, research, innovation, medical advancements and progress, we must address the challenges highlighted in the Draghi, Letta, Heitor and Niinistö reports.

According to Mario Draghi's report, Europe faces three key challenges. First, a lack of focus - Europe sets common objectives but lacks clear priorities and coordinated actions. Second, wasted resources - collective spending power is diluted across various national and EU instruments without effective coordination. Third, inadequate coordination: Europe needs more strategic alignment in policies, particularly where both small and large scale actions can support common goals and should reduce regulatory burdens in certain areas. To address these, Europe must accelerate innovation, secure significant investments, and reform EU governance to enhance coordination and reduce red tape. Strengthening public R&I collaboration across Member States is vital.

Letta's report proposes the "5th freedom" to enhance the EU's Single Market by adding research, innovation, data, skills, knowledge, and education to the existing freedoms. Key suggestions include empowering research infrastructures, creating a European Knowledge Commons, removing barriers to knowledge sharing, fostering public-private partnerships, increasing researcher mobility, championing open science, safeguarding researcher autonomy, and addressing the investment gap in R&I. Letta also recommends an European Guarantee Fund, a financial tool for solidarity that aims to translate the medical innovation achieved by the Orphan Regulation into real-world, equal access for every rare disease patient, regardless of their location in the EU. The recommendations aim to strengthen European integration and promote innovation, science, and digital literacy through coordinated policy initiatives and infrastructure improvements.

The **Heitor report** emphasises the need for a holistic, whole-of-government approach to align transformative research and innovation policies, aiming to make Europe globally competitive, secure, sustainable, and resilient. This includes increasing the investments in Research and Innovation, and delivering excellent research, impactful innovation, and technology scale-ups

through a stronger framework programme. The report proposes four interrelated action areas: competitive excellence, industrial competitiveness, societal challenges, and a robust research and innovation ecosystem. To adapt to a rapidly changing science and innovation landscape, it suggests establishing an experimental unit for testing new programme with quick funding, strengthening competitive excellence, and stimulating industrial RD&I investment. The report calls for radical simplification, user orientation, and efficiency, alongside developing an innovation procurement program to accelerate industry scaling. Additionally, it advocates for a nuanced approach to international cooperation in a complex geopolitical environment and recognises the natural dual use of modern technology.

The **Niinistö report** emphasises the need for better cooperation between Member States in health security and preparedness, urging the EU to embrace deep integration across all sectors to overcome the existing fragmentation and information gap due to a lack of relevant and reliable data in critical sectors. It calls for a transition to a "whole-of-society" and "all-hazards" security mindset. The report specifically advocates for integrating Europe's supply chains and boosting strategic European manufacturing capacity (for critical medicines) to reduce external dependencies. By integrating planning, intelligence, and coordination mechanisms and streamlining decision-making, the EU can overcome its current institutional fragmentation and achieve the speed and unity necessary to deter threats and respond effectively to complex, cascading crises.

2. A situation that demands action

Fragmentation among Member States continues to hinder the full and equitable implementation of advances in rare disease care and research. Today, this challenge is further compounded by a rapidly changing global context: geopolitical instability, the aftermath of the pandemic, and increasing pressure on Europe's competitiveness, all of which risk widening existing inequalities in screening, diagnosis, treatment and access to expertise for people living with rare and complex diseases across Europe. It is deeply concerning that the average time of diagnosis is nearly five years from the first onset of symptoms to a confirmed diagnosis for rare disease patients, with variations based on demographic and geographic factors.

We highlight the critical gap in established mechanisms and incentives necessary for optimising cross-border collaboration and facilitating cross-border access to care, particularly in the context of the European Reference Networks (ERNs). These demands tackling two pivotal challenges: (a) developing clear, standardised, yet system-adaptable service-delivery models for rare diseases, and (b) establishing robust mechanisms and platforms for cross-health system.

Translational health research should be the primary focus for addressing the increasing burden in rare and complex diseases across Europe. From a policy perspective, the translational health research continuum should be strengthened. The EU strategy should be revisited to prioritise prevention, early detection, and improved treatment. Priorities should include increasing cure rates and enhancing patients' quality of life, especially for those with incurable diseases. This requires long-term, sustainable funding of biomedical research and innovation across Europe. This is critical to avoid the traditional, fragmented "short-term, project-based funding" structure of the European Commission's collaborative research, which relies on many small projects dispersed across varied institutional contexts. Articulation with frontier research in Europe, supported by the European Research Council (ERC), and with disruptive innovation, including that sponsored by the European Innovation Council (EIC), becomes paramount.

We observe with apprehension the growing decline in Europe's competitiveness in health re-

search and innovation compared to global leaders such as the United States and China. Over the past decade, Europe's share of global research and development investment has steadily decreased, with more companies choosing to conduct research and development outside the EU. Persistent fragmentation, slower regulatory procedures, and limited investment in infrastructure and innovation ecosystems have weakened the continent's ability to attract and retain cutting-edge industries.

We reaffirm our concern over the decline in the number and competitiveness of clinical trials conducted in the EU. Over the past decade, Europe's share of global trials has steadily decreased as more research shifts to the United States and Asia. Complex regulatory requirements, lengthy approval timelines and fragmented implementation across Member States continues to deter investments and slow patient access to innovation.

We are concerned by the persistent disparities in patient access to innovative medicines across Member States. Less than 50% of centrally approved innovative medicines are available to patients across Europe, with a concerning decline in those fully reimbursed. The average time from approval to patient access has now stretched to 578 days, with significant variations seeing patients in some Member States waiting over seven times longer than others.

We emphasise that orphan medicines continue to face significant challenges in the EU, these include, limited data packages, complex value discussions, and low sales volumes, which often makes it difficult for developers to recover costs and sustain market availability. As a result, we have seen that not all orphan medicines come to Europe and cases of approved therapies withdrawn from the market over the past two decades, leaving patients without access to treatments that once addressed critical unmet needs.

We point out that manufacturers of orphan medicines face growing barriers to maintaining market access, as the high costs of clinical data generation and payer fragmentation are often disproportionate to the limited sales potential. Whereas the regulatory entry is simple thanks to the European Medicines Agency, decisions on pricing and reimbursement remains a member state competency. The HTA Regulation, that came into force early 2025, singles out orphan medicines (and cancer medicines and advanced therapies). The majority of such innovative products are developed by small to mid-sized companies, which includes many that are based outside the EU. Such companies that wish to place their products on the European market will require additional support to navigate the new requirements, due to unfamiliarite with the European market. Therefore, additional support for smaller companies similar to that which is provided by the EMA SME office should be considered, to avoid companies choosing to not enter or withdrawing products from the European market, resulting in a loss of essential diagnostic and therapeutic tools for patients and families across Europe.

We are concerned that the ERNs, despite their proven value in improving diagnosis, care and research for rare and complex diseases, continue to face major structural and financial challenges: limited and unstable funding, fragmented integration into national healthcare systems and the absence of a clear long-term governance framework threaten their sustainability and capacity to deliver on their mission.

We underline the persistent gaps in rare disease registries, the challenges in sharing high-quality data across borders, along with limited interoperability, inconsistent data standards and heavy administrative burdens. All these continue to hinder researchers and clinicians' ability to generate comprehensive insights, advance innovation and make efficient use of existing data resources

across the EU. We also note that fragmented evaluation and access pathways for safe and effective digital health technologies, including medical device software and AI-enabled tools, continue to hinder their timely, equitable and appropriate adoption across Member States. In parallel, the lack of harmonised EU-level rules for telemedicine applications limits the capacity of the ERNs to deliver remote, cross-border, multidisciplinary and highly specialised consultations at scale, despite their demonstrated impact. These gaps constrain the effective systemic integration of digital innovations at scale, that could strengthen early diagnosis, coordinated care and access to expert knowledge for people living with rare and complex diseases.

We highlight the limited coordinated European action to address the persistent skills gap, with many healthcare professionals still lacking sufficient exposure and training in rare diseases, leading to delays in diagnosis and treatment for patients, as well as to relevant health innovation applications. At the same time, complex administrative procedures, limited funding and uneven recognition of qualifications hinder cross-border cooperation, opportunities for exchange and capacity building.

We note with concern that the absence of a dedicated, earmarked health programme in the 2028–2034 Multiannual Financial Framework (EC No 2025/570) risks diluting health priorities, including rare diseases and stress the need for a robust EU Action Plan on rare diseases to build on the success of the ERNs and address existing gaps in diagnosis, treatment access and research coordination.

We observe that the European Union faces a critical juncture that demands an immediate and decisive review of its approach to securing leadership in the research and innovation of orphan therapies. Current complexities of its pricing and reimbursement framework and short-term financing threaten to push vital research and investment outside the block, eroding the EU's competitive edge and, more importantly, delaying patient access.

This urgency is amplified by key global moves: the UK's recent simplification of its regulatory environment for orphan medicines is designed to attract development, while the FDA's announcement of its new Plausible Mechanism Pathway provides a streamlined, flexible route to approval, setting a new global standard for scientific reliance.

A proactive and rapid review of the EU's regulatory, pricing and reimbursement, and incentive framework is essential. We must streamline the development and delivery of therapies and the pan-EU infrastructure needed for bench-to-bedside care, including patient representatives and advocates in the decision-making process. This is not merely an economic necessity to avoid competitive disadvantage; it is an ethical mandate to ensure European citizens, particularly those with rare diseases, are the first beneficiaries of breakthrough innovation, not the last.

The existing barriers among the 27 Member States — including divergent interpretations of legislation and heterogeneous ethics committee standards, which may be adequate for research conducted at the national level — are insufficient to ensure the timely and efficient implementation of clinical trials carried out across multiple countries, the development of market-ready products, and other essential research efforts in the field of rare and complex diseases.

THE DECLARATION'S ROADMAP FOR PRIORITY ACTION

The Declaration provides a strategic Roadmap for Priority Action to revitalise the innovation and care ecosystem. It serves as a critical document that mobilises key stakeholders to re-establish the EU's pivotal leadership role in rare diseases and life sciences globally.

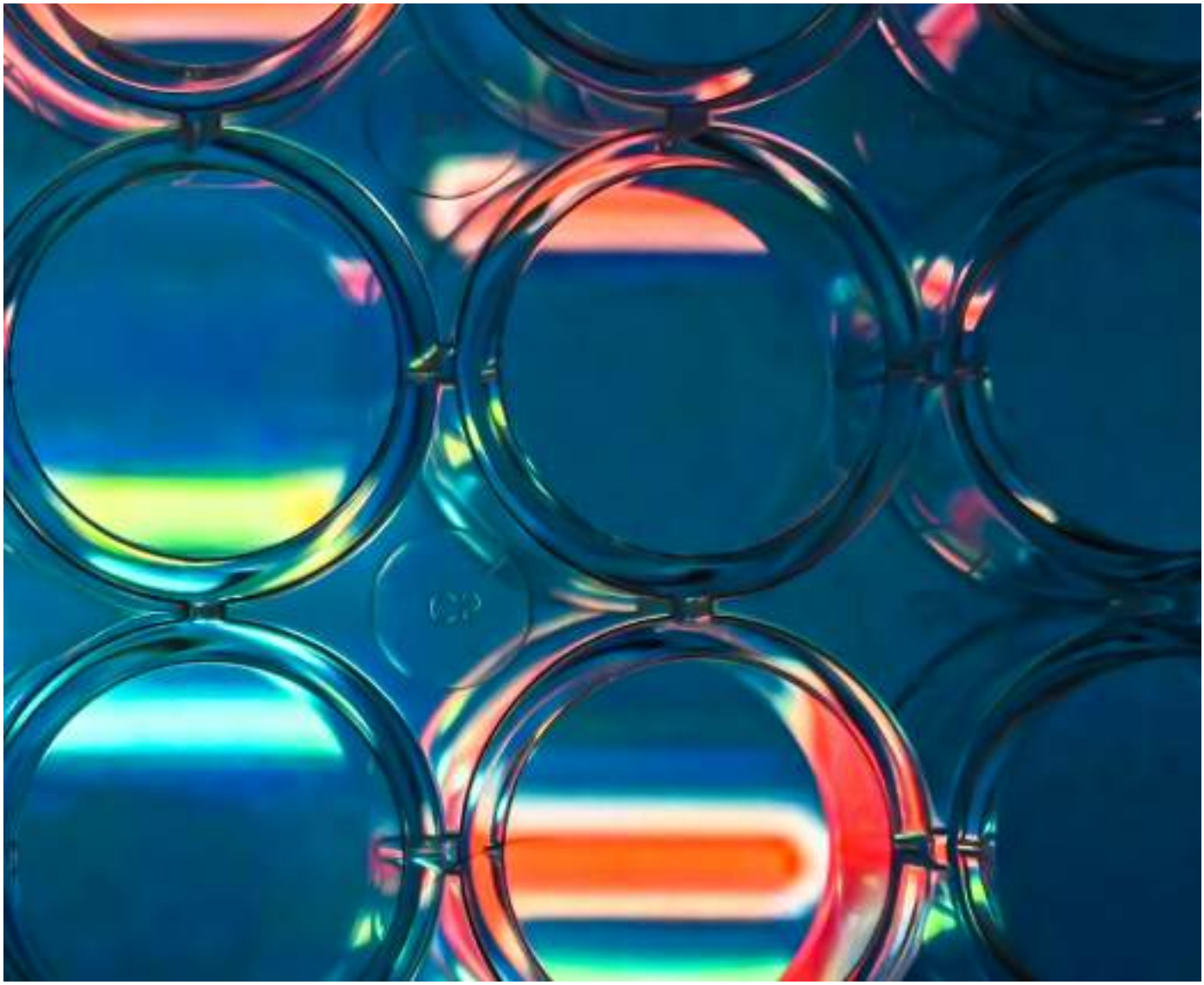
As innovation is core to this Roadmap, the contributors to this document consider a broad definition, which is understood as the invention, adoption, and diffusion of any new or significantly improved product, process, or care model, encompassing everything from advanced diagnostics and pharmacological breakthroughs to the application of cutting-edge technology and novel methods of clinical delivery, that measurably improves patient outcomes and addresses unmet needs.

We must move beyond fragmented efforts and establish a pan-European supranational ecosystem, with the ERNs as its cohesive and fully empowered foundation for innovation and care delivery across the Union. This ecosystem must align with the health systems of all 27 Member States, Iceland, Liechtenstein, Norway, and Switzerland, and create green corridors and pathways for patients. Our collective goal is to realise the vision of the EU as a competitive and sus-

tainable life-sciences powerhouse — a leader driven not only by scientific excellence, but also by an unwavering commitment to equity and solidarity for every citizen.

Translational health research should be the primary focus for addressing the increasing burden in rare and complex diseases across Europe. From a policy perspective, the translational health research continuum should be strengthened. The EU strategy should be revisited to prioritise prevention, early detection, and improved treatment. Priorities should include increasing cure rates and enhancing patients' quality of life, especially for those with incurable diseases. This requires long-term, sustainable funding of biomedical research and innovation across Europe to ensure problem-solving institutions are held accountable. This is critical to avoid the traditional, fragmented "short-term, project-based funding" structure of the European Commission's collaborative research, which relies on many small projects dispersed across varied institutional contexts. Articulation with frontier research in Europe, supported by the European Research Council (ERC), and with disruptive innovation, including that sponsored by the European Innovation Council (EIC), becomes paramount

This Declaration sets out a political vision and a structured dialogue to inform decision-making and intends to support the work of the ERN Board of Member States, which needs to be further strengthened, EU and national institutions to achieve real progress on rare disease research, development and care across the EU.



The declaration's roadmap for priority action

All the following eight priority actions are deemed equally crucial and necessitate a more coherent political framework, supported by dedicated, earmarked resources:

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

Lead the implementation of the WHO Resolution on Rare Diseases through the adoption of a comprehensive EU Action Plan on Rare Diseases, anchored by a cross-cutting Rare-Disease Mission to enable a strong European ecosystem. We recognise the necessity of immediate action for the creation of a high-level Consultative Group. This group will serve two essential functions: first, to assess the impact and coherence of the rare disease regulatory framework, verifying its effectiveness and relevance. It should focus on optimising the patients' rights Directive in cross-border healthcare and reducing regulatory overlaps of existing or soon-to-be adopted legislative acts, which can have a significant impact on research, innovation and care in rare diseases. The second function will be to develop and operationalise a comprehensive dashboard complete with clear indicators, targets, and metrics, ensuring our approach remains strictly focused on delivering tangible results.

The high-level Consultative Group should involve relevant EU Commission Directorate-Generals, European Parliament Committees, Member States representatives, the ERNs, rare disease community and industries, to ensure a robust and unified approach to simplify the regulatory environment and improve policy implementation. Instrumental for the success of the EU Action Plan would be systematic involvement of the 24 ERN coordinators, patient representatives within the ERNs, and patient advocacy groups.

Strengthen workforce development and capacity building for rare diseases and formalise the ERN academy

To retain and attract talent in rare disease research and care, it is necessary to recognise the ERN Academy and expand EU-level training and knowledge exchange initiatives to strengthen ERN collaboration and research capacity, and skills in the effective use of digital and AI-enabled applications for rare diseases.

In alignment with President Ursula von der Leyen's "Choose Europe" initiative, it is important to integrate a rare disease curriculum into medical and nursing education, championing the transformative power of the "Fifth Freedom", i.e. according to Letta's report, the free movement of knowledge, research, innovation and education in the EU Single Market.

Capacity-building and training for the health workforce specialised in rare diseases should be supported by increased EU and national funding for education and research, thus creating an environment that allows the EU to attract global talent.

Equally, with the social care dimension in mind, capacity-building for patients with rare diseases, often children, and their families must be supported.

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

We call for accelerated and equitable access to early diagnosis across the EU, including through the advancement of newborn screening programmes based on evolving scientific evidence and consensus-driven best practices. We invite the upcoming Presidencies of the Council of the EU to support the development of Council Recommendations that ensure appropriate and well-designed newborn screening programmes for rare and complex diseases, respecting both scientific advancement and ethical considerations.

We recommend an EU-level action to support the aligned collection and analysis of rare disease data, the harmonised assessment of digital and AI-enabled applications, and their responsible use to enhance early diagnosis, strengthen research and development, and improve care along the full care continuum, thereby reinforcing integrated care pathways across Member States

Foster EU leadership in clinical trials for rare diseases through inclusive collaboration between Academy, ERNs, patient groups and industry to accelerate innovation for people living with rare diseases

Creating an environment favourable to attract clinical trials in the EU is of utmost importance to address unmet needs and to revert the worrying trends of losing competitiveness against the US and Asia in clinical development of new therapies. It is therefore imperative to incentivise Public-Private collaboration involving academic research units to bring from bench to bedside innovative therapies to patients living with rare diseases.

Equally critical is accelerating the translation of trial results into clinical practice through close collaboration between the ERNs and the regulatory agencies, such as the EMA, HERA and ECDC; early implementation of validated treatments, reducing the evidence-to-practice gap and by facilitating timely adoption of proven therapies across all Member States.

To orient clinical development in areas with unmet needs, it is crucial to establish a funding mechanism that rewards these partnerships, supports rapid dissemination and implementation of positive trial outcomes, and harmonises ethical review processes across Member States to reduce time to patient access and make the EU more attractive for international investment. Involvement of patients and patient groups is vital for the development of the research strategy and implementation pathways in rare and diseases.

The EU should adopt a risk-based regulatory approach, in line with practices in the US (30 days) and UK (41 days). This must include a fast-track approval pathway for rare-disease clinical trials, which currently exceeds 110 days, recognising that lengthy timelines disproportionately affect patient access.

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

Comprehensive Rare-Disease Infrastructure Clusters (CoRDIC) are the next essential step since the foundation of the ERNs. They should embrace and bundle the needed resources for research, innovation and care for rare disease patients: translational research facilities, advanced diagnostic laboratories, the necessary human resources to facilitate early access to diagnosis and state-of-the-art care. Infrastructure for clinical trials, with capacity to run Phase I to IV trials should be included.

For reasons of equity each Member State should invest in at least one such structure if not yet existing. CoRDICS should be conceived as lighthouse nodes with an easily identifiable access point for rare disease patients to facilitate their diagnostic and care pathways as needed.

It is crucial to make urgent efforts ensuring interconnections and interoperability of existing at MS levels bio-banks and bio-registries, 1+Million Genomes (1+MG) initiatives (newborn screening data's) clusters, MS research consortia and infrastructure, AI factories as well as centres of excellence of the ERN's, thus creating effectively functioning pan-European ecosystem within MS and between MS in the EU. This task requires consistent and permanent financial support.

Boost the generation of real-world evidence by ensuring that rare-disease data are high-quality, interoperable, and clinically useful, to accelerate biomarker discovery and support drug development in rare diseases within the European Health Data Space

Ensure coordinated, end-to-end data collection and governance across ERN registries, biobanks, and structured extraction from clinical electronic health records (EHRs), following FAIR principles. Strengthen data quality aligned with the EMA framework through harmonised standards and shared validation metrics, while promoting clinical utility through integration of real-world data and PROMs co-designed with patient communities.

Deploy central and federated analytics and common data models to enable privacy-preserving cross-border collaboration, trustworthy access and robust Artificial Intelligence/Machine Learning development.

Incorporate EU-wide patient referral systems to support timely diagnosis and access to innovative treatments. Ensure that integrated, high-quality datasets across Europe effectively inform clinical care, research and regulatory pathways within the EHDS.

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

Strategic focus must be placed on developing financially sustainable early access models that unlock access to orphan diagnostics and therapeutics. This requires maintaining a continuous, open, and outcome-oriented dialogue with the pharmaceutical and diagnostic industries on practical and effective access mechanisms.

The aim should be to develop early access frameworks, considering pan-European pathways and cross-country collaboration with industry in procurement and reimbursement, thus enabling conditions that guarantee speed, continuity and coordination across borders and systems for rare and complex diseases patients, all guided by transparent, value-based, quality-driven, and patient-centred criteria.

To address structural inequities caused by varied national resources, which often deny citizens in smaller countries timely access to life-saving innovation, we urge the consideration of piloting a European Guarantee Fund, outlined in the "Much more than a market" - testing its operation on a small-scale involving Member States with limited fiscal capacity and small patient cohorts - to assess its effectiveness and feasibility.

In the realm of rare and complex diseases, it is clear that no single country can tackle these challenges alone.

Ringfence ERNs funding under the 2028-2034 Multiannual Financial Framework

To secure the ERNs' long-term sustainability and impact, it is fundamental to:

1. Ringfence funding for the pan-European innovation and care ecosystem for rare and complex diseases, as well as for the ERNs and rare disease actions in the 2028–2034 Multiannual Financial Framework (MFF).
2. Secure continuity across funding programmes, linking Horizon Europe's and EU4Health's successors for ERN coordination, and national co-funding mechanisms for ERN healthcare providers, to maintain steady financing for coordination, clinical operations, also supporting early access schemes, knowledge sharing, data infrastructure, and capacity building.
3. Transition from short-term projects to durable frameworks, such as:
   - Multi-country consortia and "infrastructure investment clubs" pooling national and EU resources.
   - EU Research Infrastructure Bonds to fund pan-European data and digital infrastructure for rare diseases.
4. Simplify administrative procedures and streamline governance between the European Commission, Member States, and ERNs to enable long-term financial planning and reduce duplication of efforts.

"For me, it is crystal clear – we need to build a stronger European Health Union (EHU)

Ursula von der Leyen -

In 2024, Ursula von der Leyen outlined the first key building blocks of the European Health Union: stronger health security measures, equal and timely access to affordable medicines for all citizens, a world-leading cancer plan, comprehensive action on mental health, rules to boost patient safety, and a One Health approach to address major health risks.

Our initiative continues to strengthen the European Health Union. While scientists, researchers, industries, and various reports all point to the need for an effective supranational EU ecosystem, which can help Member States solve their problems in health on the ground, we are not taking the necessary steps to create and implement it. We already have a wealth of components in place, yet we lack governance, finances, coordination, and clear missions that would allow these components to function together as a cohesive system. The only notable exception is the European Reference Networks, which if appropriately supported will improve their mission as a driving force in our efforts to improve health care and research.

Integrating and connecting the ecosystem on rare and complex diseases can have significant societal impact, representing a blueprint for rare diseases.

The EU vision is to make Europe the global beacon for Life Sciences by 2030.

Supported by numerous Members of the European Parliament, the signatories of this Declaration affirm our unity and commitment to advancing progress on rare and complex diseases to fully realise the European Health Union. We formally request that the European Commission, European Parliament and the Council of the EU grant the mandate to fully explore and deploy the Roadmap for Action outlined in this Declaration. Demonstrating the proof-of-concept for the EU Innovation and Care Ecosystem within the highly complex landscape of Rare and Complex Diseases will establish a scalable model of care with transformative societal impact, shaping healthcare across the EU. This Ecosystem represents a cornerstone of Europe's security, competitiveness, prosperity and global leadership.

We call on the Cyprus Presidency and the upcoming Presidencies of the Council of the EU - starting with the trio Presidency of Ireland, Lithuania and Greece - to adopt concrete Recommendations and/or Conclusions that will advance coordinated action on rare and complex diseases and keep this policy area high on the EU Political Agenda.

SIGNATORIES

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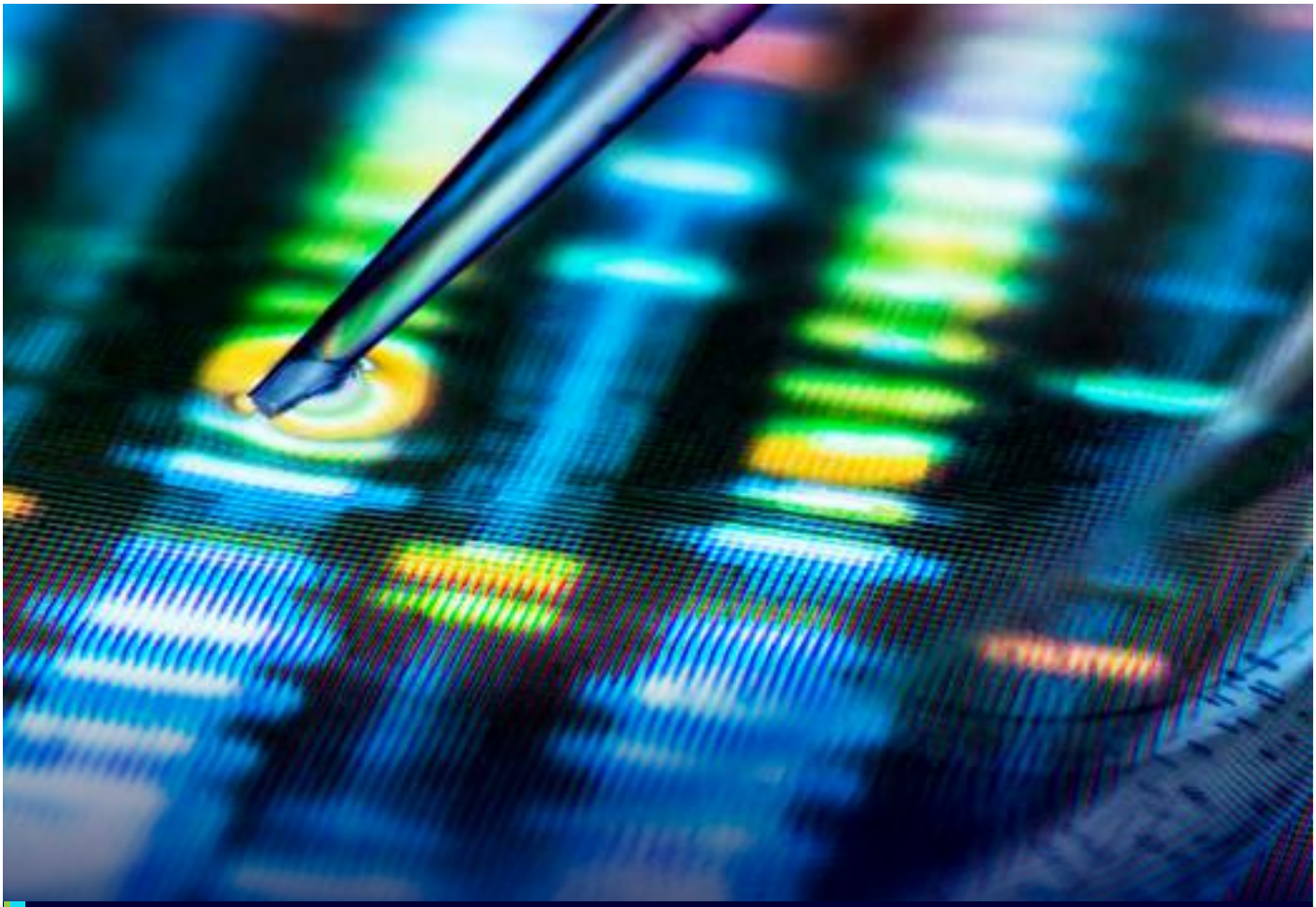


ANNEXES

1. Progress in the area of rare diseases in the EU has been achieved through numerous EU programmes

We are not starting from scratch. By recognising that rare diseases collectively affect approximately 30-36 million people in the European Union, and that patients and their families often face significant challenges in diagnosis, care and access to innovative treatments, requiring urgent and coordinated action to ensure equitable health outcomes, we acknowledge the European Commission's sustained leadership in advancing rare disease research and policy over the past two decades.

This includes landmark legislative initiatives such as the Orphan Medicinal Products Regulation (EC No 141/2000), and over €3.2 billion invested in more than 550 collaborative projects through Seventh Framework Programme (FP7), Horizon 2020, and Horizon Europe 2021-2027.
The Commission has also supported the development of patient registries, the European Reference Networks (ERNs) in 2017 under Directive 2011/24/EU on the application of patients' rights in cross-border healthcare, with the objective of improving access to highly specialised expertise and fostering cross-border collaboration among healthcare providers for rare and complex diseases. The ERN Board of Member States was established for the implementation of this Directive, specifically regarding ERN governance, bringing together Member States to steer the networks and providing a forum for collaboration on their integration into national health systems.

The Commission launched initiatives like the European Rare Disease Research Coordination and Support Action consortium (ERICA) to strengthen the research capacity of the ERNs, recognising their pivotal role in identifying unmet research needs across the rare disease community.

The 1+ Million Genomes (1+MG) initiative - an ambitious, large-scale, cross-border project that aims to enable secure access to genomic data and corresponding clinical information across Europe, which is critically important for rare disease therapies. Through the EU4Health Programme and the European Rare Disease Research Alliance (ERDERA), in October 2024, co-funded by Member States and the European Commission under Horizon Europe with over €350 million have been mobilised to strengthen care, data infrastructure, and innovation.

Today, the 24 ERNs cover the main clusters of rare, complex and low-prevalence diseases and include 1,606 specialised centres located in 375 hospitals across 27 Member States and Norway. The 24 ERN-associated patient registries demonstrate their continued commitment to systemic, standardised data collection and generation of real-world evidence to strengthen research collaboration. The Clinical Patient Management System (CPMS), operational since 2017, has enabled the discussion of more than 4,500 complex cases, enabling cross-border virtual consultations and improving access to expert knowledge. Between 2017 and 2022, more than two million of new patients were diagnosed and treated by ERN healthcare providers, underscoring the networks' growing impact on patient outcomes. The specialised knowledge and protocols developed by ERNs are essential not only for diagnosed rare diseases but also for all complex conditions, setting high standards for care pathways across the EU. The clinical centres forming the ERNs, in their daily practice, contribute significantly to shaping healthcare pathways for a wide spectrum of rare and complex diseases. Beyond direct clinical care, the ERNs have also played a pivotal role in raising visibility and awareness of the unmet medical needs and challenges faced by people living with rare and complex diseases across Europe.

We welcome the fact that the European Commission co-funds the JARDIN Joint Action, designed to serve as a bridge between ERNs and national health structures.

We welcome the adoption of the European Health Data Space (EC No 2025/327) as a landmark step towards securing access and sharing of health data across the EU. This initiative strengthens the Union's capacity for research and innovation, particularly in the context of the Apply AI Strategy, supporting the use of high-quality health data and AI-driven solutions that improve patient care and accelerate medical progress. Given the low prevalence and dispersion of rare diseases, the European Health Data Space also provides the EU-level framework for harmonised data pooling that is essential to enable trustworthy and unbiased digital and AI-enabled applications supporting earlier diagnosis, research and enhanced care. We also welcome the Digital Omnibus Regulation, which strengthens coherence and simplification across the EU's digital governance framework, recognising that streamlined and harmonised rules are key enablers of competitiveness and cross-border innovation.

We highlight the Rare2030 Foresight Study, led by EURORDIS with the support of the European Commission and European Parliament. Through a participatory process involving over 200 experts, this study analysed trends and developed future policy scenarios, thereby establishing a crucial foundation for requesting a new EU Action Plan on Rare Diseases to improve the lives of affected patients across Europe.

While acknowledging the adoption of the US orphan drug Regulation (1983), we recognise that the EU showed leadership with the adoption of the Orphan Medicinal Products Regulation (EC

No 141/2000), setting a global benchmark later matched by other regions. This global momentum underscores the need for the EU to continue evolving its approach to remain competitive and responsive to the needs of people living with rare and complex diseases. We recognise the valuable ongoing work in rare diseases conducted by EU institutions, Member States, and the broader stakeholder community. This includes the important collaborative initiatives led by EURORDIS, including with industry trade associations EFPIA and EUCOPE. The achievements and specific past initiatives being acknowledged are detailed in the Annex to this Declaration.

We welcome the growing international momentum to prioritise rare diseases within global health policy. We commend the adoption of the first-ever United Nations General Assembly Resolution on rare diseases in December 2021, which aimed to address the challenges faced by persons living with a rare disease and their families and called for inclusive, rights-based responses. We also recognise the adoption of the World Health Assembly resolution in May 2025, titled "Rare Diseases: a global health priority for equity and inclusion," which urges Member States to integrate rare diseases into national health strategies, strengthen diagnosis and care through universal health coverage, promote inclusive policies, and accelerate research and access to very much needed treatments. In addition, we support the work of the International Rare Diseases Research Consortium (IRDiRC), which fosters global scientific collaboration and drives innovation in rare disease research, and the efforts of the EU Member States in drafting and implementing their National Plans for Rare Diseases to concretise actions to answer the request of health by people living with rare diseases.

We recognise that the Council Recommendations on an action in the field of rare diseases (2009) was the first EU overarching strategy for rare diseases and a crucial step toward encouraging EU Member States to develop and implement national plans for rare diseases aiming to improve diagnosis, treatment and care of people with rare diseases.

2. Legal grounds and ongoing legislative acts

Our initiative is based on solid legal ground of the Treaty of Lisbon:

- **Article 5 of TEU** enshrines the principles of subsidiarity and proportionality.
- **Article 4.2.(k) & 9 of TFEU and Article 35 of the Charter of Fundamental Rights of the European Union** guarantee a high level of human health protection in all EU policies and activities.
- **Article 114 of TFEU** sets up the establishment and functioning of the internal market.
- **Article 168 of TFEU** ensures high level of human health protection in the definition and implementation of all Union policies and activities.

Also, on:

- **Directive (EU) 2011/24 on the application of patients' rights in cross-border healthcare** enshrines the European Commission's support of Member States in the development of European reference networks.
- **Directive (EU) 2014/24 on public procurement** sets up 30% price reduction mechanisms.
- The **2030 Agenda for Sustainable Development** (2015) pledges to @Ensure healthy lives and promote well-being for all at all ages.
- The **European Pillar of Social Rights** (2017) proclaimed that 'Everyone has the right to timely access to affordable, preventive and curative health care of good quality.'

- **Regulation (EU) 2021/2282 on health technology assessment** establishes a support framework and procedures for cooperation of Member States on health technologies at Union level.
- The **European Health Data Space** (2025) secures access and sharing of health data across the EU, creating a Single Market for digital health services and products while maintaining strict compliance with EU data protection rules, such as the GDPR.
- The **Competitiveness Compass for the EU** (2025) establishes competitiveness as one of the EU's overarching principles.
- The **Choose Europe for life sciences Strategy** (2025) aims to position the EU as the world's more attractive place for life sciences by 2030.
- The **European Preparedness Union Strategy** (2025) complements the proposed **Critical Medicines Act** (2025) to strengthen EU's resilience against public health threats, bolstering medical countermeasures and supply chains.
- The **Apply AI Strategy** (2025) focuses on competitiveness and technological sovereignty by accelerating the adoption of artificial intelligence across strategic sectors in the EU.
- **The Union of Skills Strategy** (2025) aims to address skills shortages in health professions by attracting and developing talent to ensure Europe's competitiveness and resilience in critical sectors.

We look forward to further advancements of key legislative proposals that aim to strengthen Europe's pharmaceutical and biotech sectors. The EU Pharmaceutical Legislation (EC No 2023/0131 and EC No 2023/0132) reform aims to improve access to medicines, drive innovation, and reinforce supply security. The upcoming EU Biotech Act, part of the EU Life Sciences Strategy, seeks to streamline regulatory frameworks and support biotechnology development across sectors. The EU Critical Medicines Act (EC No 2025/102) focuses on safeguarding the availability of vital medicines and improving access to treatments of common interest, including those for rare diseases, and suggesting more collaboration between member states to negotiate prices and secure access to medicines in all countries. Together, these initiatives form a robust framework to enhance Europe's health resilience and accelerate the delivery of innovative treatments to patients.

We welcome the upcoming European Biotech Act, set to bring biotech from the laboratory to the factory and onto the market. The EU Biotech Act aims to simplify EU regulations, enhance competitiveness, and support innovation in the biotechnology sector across Europe, which is particularly vital for fostering the research, development, and availability of therapies for patients living with rare diseases.

Europe is at a decisive moment for building a strong, patient-centred innovation and research ecosystem with more cooperation between Member States to ensure therapies that have been developed are accessible throughout the Union. The Commission has outlined a clear vision for fostering innovation, supporting life sciences, and improving health outcomes for millions of patients, particularly those living with rare and complex diseases.

3. Statements made by the European Commission officials

"This must be Europe's Independence Moment. […] The EU must equip itself with a modern, flexible and well-funded budget."

Ursula von der Leyen
(President of the European Commission)- in her speech at the European Parliament – Commission Work Programme 2026

She also added

- We will also focus on unleashing the full potential of the Single Market by 2028 – removing barriers in capital, energy, services and telecoms, and enabling a "fifth freedom" for knowledge and innovation through the European Research Area. We will make doing business and accessing finance in Europe easier for all companies, not least innovative companies, startups and SMEs. We will establish a European Innovation Act and set up the 28th regime for all companies operating across the Single Market, as well as putting forward the remaining proposals to complete the Savings and Investment Union.
- From recent initiatives like Choose Europe and the start-up and scale-up strategy, to upcoming actions like the visa strategy, we will make Europe more attractive for talented professionals and innovative entrepreneurs, while reducing administrative barriers.
- The proposed EUR 2 trillion Multiannual Financial Framework for 2028 to 2034 will support competitiveness, decarbonisation, security, cohesion and Europe's global mission.
- Complacency is not an option. We must act with unity, courage and conviction to strengthen our competitiveness, lead in clean and digital innovation and ensure our collective security.
- Europe has shown time and again that it can adapt, act quickly and stand united. By deepening cooperation across all levels – from EU institutions to local communities – we can ensure that our Union remains strong, sovereign and ready for the future.

"The Single Market remains Europe's best industrial policy tool: to attract investment where Europe wants to lead."

Teresa Ribera
(Executive VicePresident of the European Commission for a Clean, Just and Competitive Transition and Commissioner for Competition)

She also added

- Our revised Guidelines will provide clear and upfront guidance, including concrete criteria on when scale can benefit the Single Market. The Guidelines will provide guidance on mergers that may support competitiveness, innovation, and resilience. They will complement our toolbox with modern metrics to assess market power, innovation, and investment.
- Competition enforcement also plays a vital role in the pharmaceutical sector where it can affect access to affordable medicines.

"The life science strategy is our response. It is a clear plan to make Europe the best place for life sciences."

Ekaterina Zaharieva
(European Commissioner for Startups, Research and Innovation) -

She added:

- In short, life sciences are Europe's lifeline.
- We propose clear actions covering the whole innovation journey.
- From research through market access to uptake. They are backed by 10 billion euros annually under the current budget. And in the next MFF, I would say health is one of the biggest winners as well.
- First on research, we are proposing a new clinical research investment plan.
- We will encourage multi-country trials. We will also support access to our excellent infrastructure. And here we are not starting from scratch.
- We will accelerate the market journey of our life science startups.
- We have a great tool at our disposal, the European Innovation Council. We will leverage its portfolio. And we will launch a matchmaking platform that will connect our startups with investors and partners.
- And our Choose Europe initiative will attract new and retained talent, helping to address the staff shortage that many of our rural areas suffer from.
- Our strength in life sciences is the result of decades of investment, vision and talent.

"If we want to lead in health and deliver quicker and better results to patients, we need funding instruments that match our ambitions. [...] My long-term goal is clear. To move from fragmented efforts to one comprehensive health ecosystem, innovative, preventive and resilient."

Olivér Várhelyi
(European Commissioner for Health and Animal Welfare) -

He added:

- If we want a new health ecosystem, we need to start with the basics, and the basics are research.
- Europe is serious about investing in health research, but the next MFF cannot follow the same structure as before.
- Thus, under the next MFF, health will stand as a key component. All the objectives we are funding today will be eligible for funding as from 2028 and onwards.
- Because Europe needs a stronger and more innovative health ecosystem, one that boosts our competitiveness and strengthens our health systems.
- If we integrate health innovation across sectors, bringing together developers and users, pharmaceuticals, medical devices, AI and big data, we can unlock a brand-new wave of therapies, technologies and care models that respond to patients' personalised needs. With the European Health Data Space, we finally have a trusted foundation for using this data across the Union. Together, data and AI have the potential to transform healthcare.

- Honourable Members, the Commission is committed to strengthening the effectiveness, accessibility and resilience of our health systems, the digital transformation of healthcare, and also boosting productivity and competitiveness by improving people's health.
- This is why we have a much more comprehensive budget.
- This is a vast proportion of the sector is related to health. Eighty percent of the biotechnology market is health related. This is precisely why innovation in these sectors is so crucial for our health care and health systems, but also for Europe's long term economic strength.
- Time is crucial here, which is why we are going to present it to you this year. It will focus on creating a more innovation-friendly environment by simplifying the rules, boosting research funding, also opening up new ways of funding, opening doors, therefore, for investments, and ensuring smoother market entry for pioneering products. And doing away with the silo approach characterised so much by the current industry.
- Pharmaceutical, medical devices, artificial intelligence, big data. We need to get rid of these firewalls. The strategy also highlights the need for moving on clinical research.
- Europe's global share in clinical trials has gone down in relative terms from 23% back in 2013 to 12% in 2023. The fact is also that we are too slow, especially on multinational trials, while elsewhere authorisations move much faster. So let's bear in mind that the place of clinical trials influences largely the market entry, the availability of the therapy fast, and the production.
- It takes on average 82 days in Europe to run a clinical trial. For your information in the UK, it's 60 in the UK. In the US, it's even shorter, around 30.
- In a multi-country trial, which should be the trial we would all want to have and promote, it's even more. It's 116 days on average, which is simply, it's not sustainable. We have to act immediately.
- So when you say that we need 28 to 36 billion euros to allocate, we have more than that. In the health budget, we have 22.6, as I said, and in the horizon only, we have set aside 20 only for the health. That is 42.6. So the health sector is the big winner of the MFF proposal. We have the budget.

All of these statements underscore a single truth: the time for delay is over. The time to align, act, and accelerate is now.

Reflections on past achievements

Initiatives led by Member States

We welcome the Council conclusions on the future of the European Health Union, which call on the European Commission to keep health as a priority and recognise both the progress made in EU-level health coordination and the ongoing challenges, such as workforce shortages, supply issues, and rising chronic disease burdens. The conclusions, dated 21 July 2024, invited the Member States and the European Commission to strengthen the Healthier Together – EU Non-Communicable Diseases Initiative by adopting a comprehensive EU-level approach to rare diseases, including a European action plan on rare diseases.

We reaffirm the need to include rare diseases in high-level discussions, as they were during the high-level conference "Towards an EU Action Plan on Rare Diseases" held on 10 April 2025 under the Polish Presidency of the Council of the EU, co-organised by the European Economic and Social Committee (EESC), the Polish Ministry of Health, and the Medical University of Warsaw. This event marked a significant moment of political and stakeholder alignment around the need for a coordinated EU strategy on rare diseases.

We acknowledge the exploratory opinion adopted by the EESC on 17 September 2025, at the request of the Danish Presidency of the Council of EU, on the role of AI and Big Data in rare disease research. The opinion highlights the potential of digital tools to improve patient pathways, enable personalised medicine, and enhance quality of life, while also recognising challenges around data privacy, bias, affordability, and accessibility. It calls on Member States to accelerate health data digitisation and adopt ORPHA codes to support cross-border data sharing and the European Health Data Space.

We congratulate the Nordic Rare Disease Summit 2025, held on 16–17 September 2025 in Copenhagen under the Danish EU Presidency, focused on strengthening cross-border collaboration, improving diagnosis and care, and advancing innovation for people living with rare diseases.

Initiatives led by the European Parliament:

We welcome the organisation of the first Preparatory Meeting (April 29th) triggering our discussions about the necessity to develop pan-European ecosystem on rare diseases. We support the integration of rare diseases into European Parliament debates and welcome the plenary exchange held in April 2025 with Commissioner Olivér Várhelyi on the need for a European Action Plan on Rare Diseases. During this discussion, Members of the European Parliament called for a comprehensive rare disease strategy that bridges gaps, addresses inequalities, and responds to unmet needs through a more systemic and coordinated approach. We acknowledge the recognition of ERNs as essential tools for cross-border collaboration and support their integration into national healthcare systems.

We acknowledge the European Parliament's continued support for improving access to medicines and fostering innovation in rare disease treatment. In its resolution of 13 December 2023 on non-communicable diseases (2023/2075(INI)), the European Parliament called for a stronger European medicines market to ensure equal and affordable access to innovative treatments and personalised medicines across the EU. We also welcome the European Parliament's decision of 4 May 2022 on the discharge of the European Medicines Agency's 2020 budget, which endorsed the waiving of fees for scientific advice to academic researchers developing orphan medicines, an important step in supporting early-stage innovation in rare disease research.

We welcome the SANT Committee's decision to commission a European added value assessment to accompany the upcoming legislative own-initiative report (INL) on a European Action Plan for Rare Diseases led by MEP Nicolás González Casares (Spain, S&D). We anticipate that the study, expected by early 2026, will provide a robust foundation for shaping a coordinated EU strategy that addresses the unmet needs of people living with rare diseases and strengthens cross-border collaboration.

We welcome the European Parliament's continued commitment to cancer and rare disease policy and commend the launch of the Intergroup for Cancer and Rare Diseases 2024–2029, marked by its constitutive meeting on 4 February 2025. We support the Intergroup's role in engaging with civil society and key stakeholders, monitoring EU actions, and building consensus for policy initiatives that promote equitable access to care and innovation.

We reaffirm the importance of integrating rare diseases and childhood cancers into the European Parliament's research and health policy agenda and welcome the European Parliament's Panel for the Future of Science and Technology (STOA) workshop held in July 2023, which focused on the EU policy framework for rare diseases. During this exchange, experts and policymakers identified key challenges in rare disease care provision and emphasised the need for stronger collaboration between Member States.

We acknowledge the in-depth analysis of the results of the European Parliament's public consultation on rare diseases, presented to the SANT Committee on 1 December 2025, as these findings provide essential insights into the unmet needs of patients, highlight systemic gaps across Member States, and offer evidence-based guidance for shaping future EU policies and coordinated action. With more than 4,000 people participating in the consultation, we recognise the strong public support for an EU action on rare and complex diseases.

Stakeholder-led initiatives on rare and complex disease

We recognise that progress in rare disease research and policy has been driven not only by institutional action but also by community-led initiatives such as the Rare Disease Moonshot and other joint actions that have shaped strategic priorities and fostered patient-centred outcomes.

We emphasise the importance of existing public-private collaborations, exemplified by projects such as Conect4Children, Screen4CARE, Share4Rare, PARADIGM, REMEDI4ALL, and RealiseD which play a critical role in advancing clinical trials, digital tools, and personalised approaches to rare disease management and clinical care. We recognise the essential role of Orphanet as a unique and authoritative resource dedicated to improving knowledge on rare diseases. Orphanet's mission is to provide a codification system for rare and complex diseases, helping to raise their visibility in health and research information systems. Established in France by INSERM in 1997 and supported by the European Commission since 2000, Orphanet has grown into a global consortium of 40 countries advancing rare disease research.

We recognise the establishment of the European Expert Group on Orphan Drug Incentives (OD Expert Group), led by a Steering Committee composed of EURORDIS, The Voice of Rare Disease Patients in Europe and EUCOPE and chaired by Prof. Maurizio Scarpa, as a vital initiative to foster dialogue and support the development and accessibility of orphan medicines across Europe.

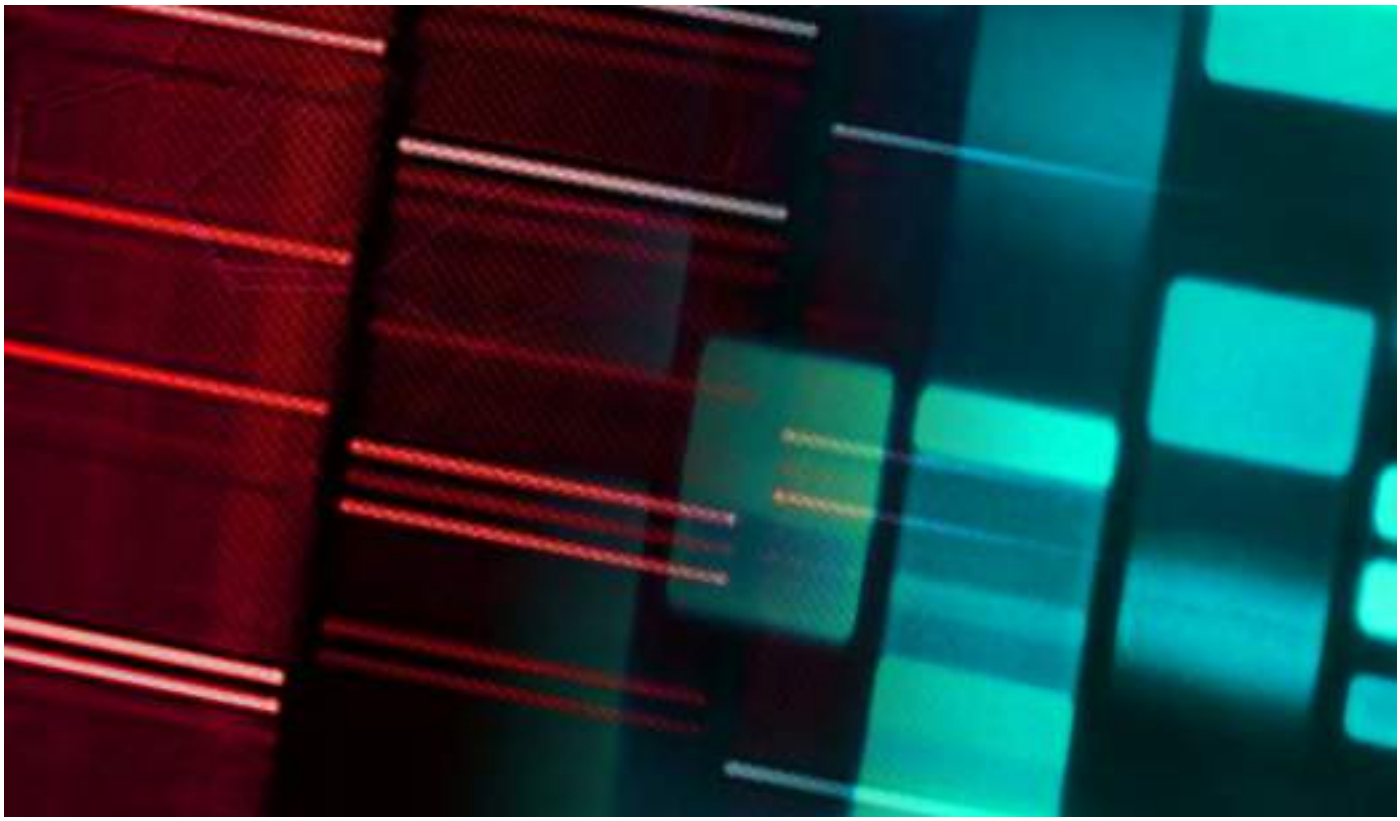
We welcome the paper prepared by M.Heitor for presentation and discussion during the High-Level meeting on the European Ecosystem for Rare and Complex Diseases. The paper discusses the need for Europe to unite its forces under the next Multiannual Financial Framework,

MFF 2028-2034, to accelerate a collective response to geopolitical threats, boost innovation in defence and security, and rethink our understanding of "science for policy" in times of knowledge abundance.

We welcome the organisation of high-level multi-stakeholder events that advance the rare disease agenda and explore its link to Europe's competitiveness agenda. We commend EURORDIS-Rare Diseases Europe and Orphanet for convening the 13th European Conference on Rare Diseases & Orphan Products (ECRD 2026), to be held in Prague in June 2026. ECRD 2026 will launch a multi-stakeholder process to co-develop a European Action Plan for Rare Diseases. We also recognise the importance of the Rare Diseases Forum 2024, organised by EFPIA, EURORDIS-Rare Diseases Europe, EUCOPE, EuropaBio and industry partners, which underscored the need to secure Europe's leadership in R&D and innovation for people living with rare diseases.

Future relevant meetings

- The **EU Conference "Advancement of Treatments for Rare Diseases"** will be held on 5–6 March 2026 in Nicosia, hosted by the Cyprus Institute of Neurology & Genetics as a flagship event of the Cyprus EU Presidency. Funded by Horizon Europe, it will gather experts, EU institutions, patient groups and industry to strengthen collaboration, promote innovation, and advance research, regulation and equitable access to treatments for rare diseases. The conference will serve as a key platform for policy dialogue and scientific cooperation, reinforcing Europe's leadership in rare-disease research.
- **13th European Conference on Rare Diseases & Orphan Products (ECRD 2026)** will be convened by EURORDIS-Rare Diseases Europe and Orphanet on 3-4 June 2026 in Prague. 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.



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