Together4RD
RARE DISEASES

– Report

Boosting EU Competitiveness through Public-Private Partnerships in Rare Disease Research

– Conference
24 September 2025
European Parliament, Brussels

– Co-hosted by:
MEP Stine Bosse (Renew, Denmark)
MEP András Kulja (EPP, Hungary)
In collaboration with
Together For Rare Diseases

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Executive Summary

On the 24th of September 2025, Members of the European Parliament **Stine Bosse** (Renew, Denmark) and **András Kulja** (EPP, Hungary), Together for Rare Diseases' MEP Champions, co-hosted a high-level conference on the urgent need for EU policy to enable ERN-industry collaboration on rare disease research.

The event was organised by Together For Rare Diseases (T4RD) and joined in-person by 40 representatives from ERNs, research infrastructures, patient representatives, the European Parliament, the European Commission's DG Research and Innovation, the pharmaceutical industry and trade associations, with 89 more registered online.

The main objective was to demonstrate how public-private partnerships (PPPs) between European Reference Networks (ERNs) and industry can boost the European Union's competitiveness by accelerating rare disease research as well as identifying the barriers and needed solutions. Public-private partnerships are delivering value and should be acknowledged as essential drivers of societal progress. They have already delivered well beyond immediate market returns whilst creating long-term value for patients, healthcare systems and society as (for example) clearly demonstrated by the many Innovative Medicine/Health Initiative funded projects.[1]

> *From the first time I heard about ERNs, I knew we had a system worth investing in.*
>
> **- MEP Stine Bosse**

ERNs are Europe's flagship rare disease infrastructure, and operate at the heart of diagnosis, patient care, registry development and clinical trials. However, while ERNs have excelled in clinical care, education, and guideline development, their research contributions have been limited or less visible (without "ERN branding"), partly because research may not have been seen as a central part of their mission as ERNs received research funding through Horizon 2020, its successor Horizon Europe and co-funds from Member States through the European Joint Programme on Rare Diseases (EJP RD) and ERDERA. For ERNs to contribute directly to European competitiveness, they must move from being the backbone of rare disease care to becoming engines of research and innovation.

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[1] https://www.ihi.europa.eu/about-ihi/impact

A change of perspective is needed to embrace public-private partnerships between ERNs and industry as flexible, well-resourced and highly productive forms of collaboration which can unlock rare disease research in Europe.

They need to be encouraged by public and private research funders. A clear signal about the expectation that some objectives are addressed by public-private consortia would be important to promote and demystify these types of collaborations. Industry are key partners for researchers, as **highlighted by ERN coordinators and industry partners in a joint T4RD and ERICA webinar** as well as by a representative of a patient organisation who shared it was easier work with industry than with ERNs.

Although several ERN-industry public-private partnerships such as Conect4Children have been highly successful, these types of collaborations are still limited by the **2019 statement by the ERN Board of Member States (BoMS)** discouraging ERN-industry data collaboration.

To fully harness the potential of PPPs, they should be allowed and promoted as effective conduits for rare disease research, notably in the ERDERA (European Rare Diseases Research Alliance) ecosystem, which already provides a coordinated EU-level framework and support for RD collaboration. Greater legal flexibility as the absence of an ERN legal entity remains an administrative hurdle, with researchers advocating for more centralised and transparent governance for ERNs.

> *Together For Rare Diseases' three ERN-industry pilot projects have demonstrated the willingness of both parties to enter partnerships to better understand rare disease burdens on quality of life, develop innovative endpoints or advance the consolidation of data registries.*
>
> *As a result, the ERN Board of Member States will look into the learnings of the pilot projects to assess the possible revision of the 2019 Statement.*
>
> **- ERN Board of Member States (BoMS)**

Europe now has a critical policy window to boost rare disease research. The Multiannual Financial Framework (MFF) for 2028-2034, unveiled by the European Commission in July 2025, **provides an opportunity to better exploit ERNs' potential by placing strategic emphasis on biotechnology**. This allows rare diseases to be woven into flagship EU funding instruments such as the proposed European Competitiveness Fund (a €409 billion initiative targeting strategic sectors including biotech) and Horizon Europe's successor programme **FP10** (with €175 billion allocated for 2028-2034). This includes ensuring that the **Life Sciences Strategy** (adopted July 2025), the forthcoming **Biotech Act**, the **European Innovation Act** (expected Q1 2026), implementation of the **EHDS** Regulation (which entered into force in March 2025), the **EU Startup** and **Scaleup Strategy** (launched May 2025), and the **European Strategy on Research and Technology Infrastructures** (adopted September 2025) are all opportunities for unlocking rare disease research and translation. The Life Sciences Strategy is one of the main strategies under the competitiveness agenda and aims to strengthen the rare disease ecosystem by improving access to data, tools, actors and partners for collaboration. An **EU Action Plan on Rare Diseases**, strongly supported by MEPs in an April 2025 plenary debate, would ensure funding and policies are efficiently directed towards boosting rare disease innovation.

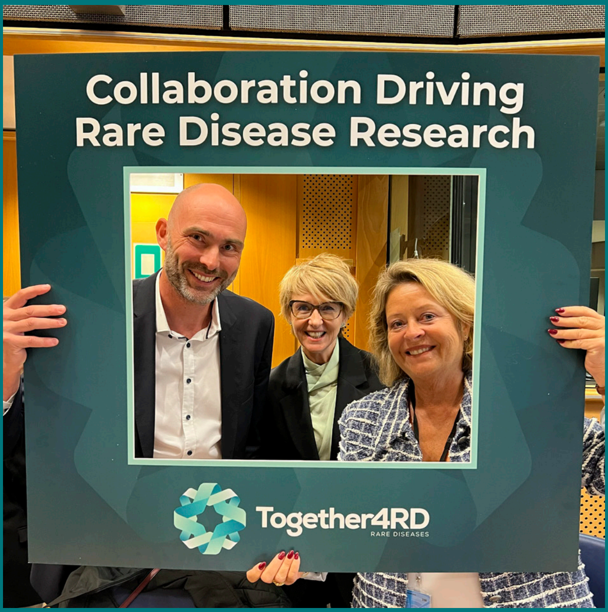
> *An EU Action Plan on Rare Diseases would be a way of attracting investment from global pharmaceutical companies and incentivise them to develop technologies here in Europe.*
>
> **- MEP András Kulja**

> *A comprehensive EU Rare Disease Action Plan is not a luxury but a necessity to bring science, solidarity and industry together for every rare disease patient in Europe.*
>
> **- MEP Vytenis Andriukaitis** (former EU Health Commissioner)

Participants called for the proposed **MFF 2028-2034 to ringfence funding for health infrastructures such as ERNs** to anchor rare diseases as a strategic, competitive investment. Suggestions for the MFF included earmarking part of the ERN core budget for clinical trial readiness and public-private partnerships, as well as establishing a network of ERN centres qualified for early human trials to accelerate patient access to advanced therapies. On the other hand, the envisaged **MFF Single Rulebook for Financial Rules,** while positive for some aspects of simplification, could impose a one-size-fits-all approach to research which will make it harder and less flexible for public-private collaborations to occur and thrive.

> *It is very hard to keep health on the top of the European agenda. As much as you need me, I need you to* ***speak up*** *and continue to talk into this obvious fact that when we work together we can do more and we can do it cheaper.*
>
> **- MEP Stine Bosse**

The momentum created by the European rare disease community has succeeded in placing rare diseases on the policy agenda as rarely before.



Together For Rare Diseases calls upon its MEP Champions and members to carry forward the optimism and suggestions surrounding public-private partnerships between ERNs and industry, and to disseminate them in key discussions such as the MEP Interest Group on Cancer and Rare Diseases[2] and the High-Level Meeting on European Research and Innovation for Rare Diseases.[3]

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[2] https://www.europarl.europa.eu/meps/en/intergroup/details/7898/Intergroup%20on%20Cancer%20and%20Rare%20Diseases

[3] https://www.brains4brain.eu/category/meeting/

Detailed learnings

Public-Private Partnerships (PPPs) as drivers of Rare Disease innovation

PPPs must be recognised as a policy tool rather than an optional add-on. Stronger political mandates and targeted funding are essential to make PPPs routine in EU health and research policy. Clear political signals must be established in upcoming legislative packages (Life Sciences Strategy, Biotech Act and the next MFF) to earmark resources and explicitly promote PPPs as a strategic instrument for Europe's health and industrial policy.

Persistent 'perceived' obstacles remain, with the 2019 Board of Member States[4] (BoMS) statement discouraging ERN–industry data collaboration for research. There is a need for a revision to clarify conflict-of-interest rules, and build trust so that collaboration is seen as safe and legitimate.

Beyond removing barriers, stakeholders called for enablers such as robust and flexible operational frameworks, standardised contracts and data-sharing agreements, and EU-level templates that can be adapted across different disease areas to lower administrative burden and speed up project start-up.

PPPs, through the generation of real-world evidence, position ERNs as pivotal hubs for trial readiness and data-driven innovation.

> *We do not only need to remove or amend the statements that prevent us from collaboration, we also need a very clear traction from funders and decision makers saying that public-private collaboration is part of our tools to achieve objectives.*
>
> **Pharmaceutical industry**

> *What we are still lacking largely are PPPs that would drive therapeutics development by exploiting the unique patient and data resources available in the ERNs.*
>
> **ERN coordinator**

> *Agile, bespoke partnerships between ERNs and industry can move the needle in rare disease research.*
>
> **Pharmaceutical industry**

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[4] The Board of Member States (BoMS): ERN governing board

Strengthening ERNs for research and competitiveness

ERNs' original mission was primarily clinical care, which means that research infrastructures, regulatory preparedness and systematic data integration have been limited. There is a need for **dedicated EU and national funding to build sustainable ERN research capacity**.

Participants called for a **ring-fenced share of the next Multiannual Financial Framework (MFF)** to support not only clinical-trial readiness and PPPs but also registry harmonisation, patient-reported outcomes development, early-phase trial capacity, and cross-border data platforms, so that ERNs can become true innovation accelerators.

New **governance models were explored**, including for the European Commission and member states to reconsider granting ERNs legal entity or creating shared governance/umbrella entities. These models would **simplify contracts, data use, and liability management, reduce administrative duplication across member hospitals, and create a single trusted entry point for industry and academic partners**.

Strengthening ERNs' research-capacities also requires training and resources for the academic centres teams (inc. on how to collaborate with private partners), interoperable IT systems, and incentives for hospitals to invest in research roles.

> *ERNs must not only remain the backbone of rare disease care, but also evolve to become engines of research and innovation.*
>
> **MEP**
> **András Kulja**

> *Creating a shared legal entity saves money and time because you harmonise and have one single entry point for the collaborations.*
>
> **Researcher**

> *Rare diseases are a cornerstone of the European Commission's health research, and the collaboration between ERNs and infrastructures like ERDERA and Jardin are exemplary for other health areas.*
>
> **European Commission**

EU policy and funding frameworks
(MFF, Life Sciences Strategy, Biotech Act)

Health must stay prominent in EU budgets, with explicit and sustained prioritisation of rare diseases. Without dedicated lines in the Multiannual Financial Framework (MFF), Europe will miss the chance to anchor rare disease research as a strategic competitiveness investment.

The competitiveness framing opens a unique opportunity to weave biotech and rare diseases into EU funding instruments such as the forthcoming European Competitiveness Fund and the next Horizon Europe work programmes. This includes ensuring that the upcoming legislative framework explicitly mention rare diseases and support translational research and advanced therapies.

These frameworks should **provide clear incentives for the co-investment from the private sector**, including risk-sharing mechanisms, tax and regulatory enablers, and predictable multi-year budgets to attract global R&D capital to Europe.

Flexibility and fitness-for-purpose are critical as concerns were raised that a proposed “single rulebook” for EU funding could unintentionally hinder diverse partnership models by imposing one-size-fits-all administrative rules. Speakers argued for **adaptable governance and regulatory sandboxes allowing different PPP formats**, lighter reporting where appropriate, and the ability to pilot innovative contracting models.

Coordination across EU directorates and member states must improve so that health, research, and industry policies reinforce each other. The group called for stronger horizontal links between DG SANTE (EU4Health) and DG RTD (Horizon) and for more structured engagement of national health ministries in EU research funding decisions.

> *What is positive about competitiveness is that it will never have been easier to talk about collaborations between industry and ERNs. There is no better time than now.*
>
> **European Commission**

> *Framework programmes are created based on the academic needs, not on the needs of public and private. That needs to change if we want to be competitive.*
>
> **Pharmaceutical industry**

> *The ERNs should no longer be denied institutional budget funding for research by the Commission, nor industry collaborations by the BoMS.*
>
> **ERN coordinator**

Building multi-stakeholder and political momentum to foster rare disease research in Europe

There was a call for strong(er) alignment between EU and national rare disease strategies, ensuring that European investments translate into coordinated national action plans and consistent funding across member states.

Participants highlighted the need to **integrate rare disease priorities into other EU policy areas** such as the digital health, data spaces, industrial, and education strategies so that rare diseases remain visible in broader competitiveness and innovation agendas.

Sustained communication campaigns and high-visibility events, such as regular summits and parliamentary hearings, keep **rare diseases in the public and political eye**, ensuring continued pressure on decision makers.

Stakeholders advocated for **ERN monitoring and evaluation to capture PPP outcomes**, including metrics on patient impact, research outputs, and economic returns, to demonstrate value and secure long-term political and financial support.

International collaboration beyond Europe, including transatlantic and global partnerships, such as IRDiRC[5], is vital for tackling ultra-rare conditions and for **positioning Europe as a global leader in rare disease innovation**.

> *As a parent, I want to see the EU not only talk but deliver an action plan so that families like mine know treatments will come to Europe, not leave it.*
>
> **Patient organisation**

> *ERNs can be the engine of change and recognising this would be a way of attracting investment from global pharmaceutical companies and incentivise them to develop technologies here in Europe.*
>
> **MEP András Kulja**

> *This is the moment to hard-wire rare diseases into the EU's competitiveness agenda, patients and their organisations are ready to work with MEPs and the Commission to make it happen.*
>
> **Patient organisation**

> **The positive aspect of this new competitiveness flag is that it has never been easier to discuss collaborations between industry and the European Reference Networks. This is an opportune moment for such partnerships**. *Biotech has been designated as a critical technology within this competitiveness framework, which represents significant progress for the rare disease ecosystem. In the current budget proposal figures provide an excellent foundation for negotiations.* **Rare diseases have frequently served as examples and catalysts for advancing other legislative initiatives**. *All the necessary instruments and tools are now available to move this agenda forward under the competitiveness framework.*
>
> **European Commission**

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[5] https://irdirc.org/

Key actions proposed by the Together For Rare Diseases initiative

Actions	Description	Stakeholders
Revise the 2019 BoMS statement to explicitly allow and encourage ERN–industry research and data collaboration	Updating this guidance would lift a major regulatory barrier to ERN–industry collaboration. By clearly allowing data sharing and joint research, it would unlock stalled partnerships and speed up clinical innovation.	Board of Member States (BoMS), European Commission (DG SANTE)
Establish an EU Rare Disease Action Plan with measurable targets and stable funding	A formal, measurable plan at EU level would provide a long-term roadmap, attract private investment, and give Member States a shared framework to align national strategies.	European Commission (DG SANTE and DG RTD), European Parliament, Member States. And all other stakeholders to continue advocating
Ring-fence part of the next Multiannual Financial Framework (MFF) for RD research, clinical-trial readiness, and PPPs	Dedicated budget lines would secure predictable financing for cross-border clinical trials, registries, and PPPs, ensuring continuity beyond short funding cycles.	European Commission (DG BUDG and DG RTD), Council, European Parliament
Create a permanent EU-level cross-sector working group to coordinate rare disease policy and monitor implementation	This forum would keep rare disease policy high on the agenda, allow stakeholders to track progress, and foster coordinated action across Commission services, Parliament, industry, and patient organisations.	European Commission (DG SANTE, DG RTD and DG GROW), European Parliament, patient organisations
Develop and deploy a standardised EU-wide contracting and data-sharing framework for ERN–industry partnerships	A coordinated and 'centralised', EU-endorsed framework would reduce legal complexity, speed up partnership formation, and ensure compliance with privacy and data-protection standards.	European Commission (DG SANTE, DG RTD and DG CONNECT), ERN coordinators, industry associations
Support ERNs in becoming legal entities or forming shared-governance structures to streamline partnerships	Legal entity or a common governance umbrella would simplify negotiations with industry, clarify liability, and enable more agile multi-country projects.	European Commission (DG SANTE), ERN coordinators, Member States
Launch a European training and mentorship programme to build collaboration skills ("collaboration muscle") among ERN leaders, patients, and industry	Dedicated capacity-building would strengthen the "collaboration muscle," helping ERN leaders, patients, and industry partners to manage complex PPPs and to innovate jointly.	European Commission (DG RTD), ERNs, patient organisations, industry
Integrate rare disease priorities into broader EU policy areas (digital health, data spaces, industrial strategy) to ensure visibility and funding	Mainstreaming rare disease considerations into digital health, industrial strategy, and education would amplify impact and guarantee sustained visibility and funding.	European Commission (DG SANTE, DG RTD, DG CONNECT, DG GROW), Member States
Establish regular high-visibility events and communication campaigns to keep rare diseases on the EU political agenda	Ongoing public engagement would maintain political pressure, demonstrate success stories, and attract new partners and investors to the field.	All stakeholders, including the European Commission (DG SANTE, DG RTD, DG GROW), European Parliament, Member States, European Economic and Social Committee, ERNs, research infrastructures, patient organisations, industry
Include PPP outcomes in the monitoring and evaluation of ERNs	Transparent indicators on patient benefits, scientific outputs, and economic returns would provide evidence of impact, helping secure future investment and public trust.	European Commission (DG SANTE), ERNs

Participants list

In-person participants (40)

Policymakers

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Participation Statistics