

# Identifying opportunities for Rare Disease research in Europe: optimising ERN-industry collaboration

Policy Workshop – report

September 17<sup>th</sup>, 2024

## Welcome and introduction

Together For Rare Diseases (T4RD) organised on 17 September an online Workshop on “**Identifying opportunities for Rare Disease research in Europe: optimising ERN-industry collaboration**”. The aim of this online interactive workshop was to analyse barriers (real or perceived) which have hampered interactions between European Reference Networks (ERNs) and Industry in the past, especially policy-related barriers, and co-develop, with participants, a practical ‘solutions implementation roadmap’ to unlock partnership opportunities, which could form one of the pillars of the 2025 T4RD action plan. After the European Parliament elections in June and the appointment of the new European Commission, the workshop provided a timely opportunity to assess the state of play in this important topic and outline concrete actions for engaging key stakeholders within EU institutions.

One important focus of discussion was the [statement](#) on the ERNs’ collaboration with industry, issued by the Board of Member States (BoMS) of ERNs. The relevance of this statement, and its impact on hindering versus fostering ERN-Industry collaborations, has been discussed in BoMS meetings, with some suggestions to clarify the statement in certain respects. The learnings gained from this workshop could serve as an important milestone in that process

## Setting the scene - the political landscape of rare disease research in Europe

The chair from the Board of Member States (BoMS) of ERNs emphasized the **critical role ERNs** play in the broader European and global rare disease research ecosystem. She highlighted the potential for public-private partnerships (PPPs) to accelerate innovation in rare disease research (RDR). She stressed that the BoMS is closely monitoring the ongoing efforts and awaiting the work of the Together for Rare Diseases initiative, to determine whether a formal statement should be reissued on the framework of such research collaborations. Her remarks highlighted the importance of the BoMS’ role in guiding and supporting these networks as they navigate the challenges of establishing sustainable research activities and partnerships through industry cooperation.

Following this, the current chair of the ERN Coordinators' Group presented his vision for the **long-term sustainability of ERNs**, particularly through partnerships with the private sector. He emphasized that clear, streamlined procedures are essential for facilitating such collaborations. He outlined the potential benefits for patients, including improved access to cutting-edge therapies and innovations. He then introduced the T4RD pilot involving his own ERN, ERN-BOND, dedicated to rare bone disorders. This is a pilot project with Sanofi, centered on real-world data collection and sharing, and the use of sensors in clinical trials. He highlighted some of the challenges the project had faced, notably in the technical and legal setup of the collaboration, whilst noting that these issues were nearing resolution. A Sanofi representative later agreed that the pilot has indeed encountered certain challenges – which is unsurprising, as after all this is exactly what the T4RD pilots were set-up to explore- and noted that while progress has been made, not all challenges have yet been fully resolved. For instance, the complexities of collaborating across different sectors presents difficulties, particularly due to varying language requirements and the rigidity of standardized templates. It was emphasised that ongoing concerns must be addressed before the project can move forward smoothly.

A trade association representative **explained what small and mid-sized companies need to consider and to improve when seeking to engage with ERNs**. He highlighted the untapped potential within Europe for driving drug development and advancing RDR, stressing that greater collaboration between ERNs and industry is crucial. He pointed out the challenges posed by conflict-of-interest rules and the importance of maintaining transparency in all collaborative efforts to ensure mutual trust and effective partnerships. He emphasized that smaller companies, in particular, could greatly benefit from better alignment with ERNs, leveraging the extensive networks and data available within these consortia to enhance research and development activities.

An EU policymaker then discussed the **position of the European Commission in fostering collaboration between ERNs and industry**. She echoed earlier sentiments about the vital role of ERNs in advancing rare disease research and outlined the challenges often raised concerning the current legal entity status of ERNs. She also emphasised the significant variety across the networks in terms of size and maturity regarding their existing collaboration with the private sectors. This heterogeneity can complicate partnerships with industry and create additional hurdles, especially for those lacking long-standing collaborative relationships. She acknowledged that while some ERNs are well-positioned to engage in such collaborations, others are still building the necessary capacity. She reiterated **the need for a coordinated effort to address these barriers and make the most of the public-private collaboration opportunities available**.

A patient organisation representative further emphasized the pressing **need to connect ERNs with both public and private initiatives** to tackle the immense challenges facing the rare disease community. He noted that 95% of rare diseases still have unmet medical needs, and called for a strategic framework that defines research priorities and partnerships to help close this gap. He also stressed that ERNs have the potential to act as a bridge between academia, industry, and patients, providing a structured platform for advancing innovative solutions to these longstanding challenges.

## Workshop objectives & approach

The overarching goal of the workshop was to assess the current state of ERN-Industry collaborations and identify practical steps to improve them, ensuring that PPPs can flourish in the RDR ecosystem.

A trade association representative addressed the importance of building trust and understanding, to help manage and address concerns that naturally arise when engaging with diverse stakeholders. Organisations frequently face challenges, either by over-regulating processes or relying too heavily on existing EU regulations, which can hinder effective collaboration, such as the BoMS statement. There is a significant need for spaces where individuals and organisations can initiate projects and collaborate more freely and yet be fully compliant with the existing legal framework regulating the collaborations between industry and academic teams. Additionally, understanding the incentives for ERNs and the industry is vital for fostering engagement and participation.

An ERDERA representative then provided insight into the broader challenges facing RDR. She pointed out that ERNs often struggle with insufficient funding, under staffing and limited research track records, which can hinder their ability to engage effectively in high-impact collaborations. She emphasized the need for a clear and robust framework to guide these partnerships, ensuring that all institutions, particularly those that are underrepresented, have an opportunity to participate. She called for more equitable involvement across the board, underscoring the importance of creating an inclusive environment that fosters innovation and advances in RDR.

Challenges	Answers
Lack of dedicated funding	21/28 (75%)
Limited ERN experience/capacity in contracting for research	18/28 (64%)
Privacy and ethical concerns	12/28 (43%)
Conflict of interest management	11/28 (39%)
Limited research track record in some ERNs	11/28 (39%)
Confusion in defining ERN research	8/28 (29%)
Perceived restrictions imposed by BoMS	7/28 (25%)
Prioritization of care over research	7/28 (25%)

The T4RD team emphasised that the results of this poll were intended purely to support discussions at this workshop. T4RD recognises that what may be a major challenge for one ERN may not be a significant challenge for another. The challenges in the poll are also very heterogenous in nature, with some warranting more fundamental policy and R&D-based solutions, whilst others call for more focused and practical action, for instance in the capacity-building space. For these reasons, the T4RD Chair assured the group that this initiative will continue to work on multiple levels, and to this end is engaging in a range of activities besides the 3 pilots (see conclusions).

Using the poll results as a launching point for discussion, the group now delved further into specific issues. One of the primary obstacles raised was the lack of dedicated funding for research initiatives. This challenge was underscored by multiple participants, who emphasized that without dedicated funding streams from either European or national sources, the research output and innovation potential of ERNs remain constrained.

Another significant issue discussed was the limited research track record within some ERNs. Many ERNs centre on disease communities which have not necessarily worked together for many years

and have not historically been very active in scientific research. They had limited opportunities, therefore, to engage with the private sector before becoming an ERN, and little has changed in the years since the ERNs' launch in 2017. Participants explained that this lack of a strong track-record can affect their credibility when applying for funding or seeking to enter into partnerships with industry. Participants also highlighted the need for ERNs to improve their research outputs and visibility, to enhance their appeal to potential funders and partners. This was highlighted to be a major need, as many in Industry are not aware of the expertise and resources ERNs can truly offer. A trade association representative and others suggested addressing this by seeking alternative funding opportunities and fostering greater collaboration within the ERN ecosystem and with external partners, both from academia and industry.

The limited experience and capacity in managing research contracts was also a central theme of the discussion. Many ERNs lack the legal and administrative expertise required to navigate complex research contracts, particularly when working with industry. Many participants agreed that more resources and training should be made available to ERNs, AND to Industry, to strengthen their respective capacity in contract management, potentially through toolkits. Interestingly, this group were supportive of the idea of using standardized templates and seeking legal guidance to help streamline these processes. When this topic was discussed in a previous T4RD workshop, to advance discussion on the content of the forthcoming T4RD Toolkit, standardised contracting templates were similarly perceived as a great need for ERNs and Industry; however, the complexity of the various types of research parties may wish to engage in, and the vast geographical spread of the ERNs, with each national rules applying, led participants especially from the Industry community to doubt the feasibility of delivering something useful and useable, in practice. This is therefore one of the more contentious potential 'tools'.

Conflict of interest management was also raised as a critical concern. Participants recognized that managing conflicts of interest, especially when industry partners are involved, is essential for ensuring transparency, maintaining trust, and safeguarding the integrity of the research. To address this, participants discussed the importance of clear guidelines and internal education to help ERNs and their partners manage these conflicts more effectively. Again though, many years of fruitful collaborations show that these issues can be, and are, managed well, and this is therefore not something many perceive to be a major barrier in practice.

A trade association representative proposed that the challenges outlined could be mitigated by encouraging greater collaboration across European and national funding sources and fostering partnerships between ERNs, academic institutions, and industry. She emphasized the importance of creating an environment where ERNs can test and develop these collaborations effectively, allowing for more innovative and sustainable research initiatives. A key point raised was the potential for public-private partnerships (PPPs) to play a crucial role in addressing some of the financial and operational gaps. Such partnerships could provide not only the funding but also the expertise needed to elevate ERN research capacity, while highlighting the different contributions of the industry in PPPs in RDR. The workshop participants emphasised that indeed, it is important for ERNs to appreciate that collaborations with Industry should bring more than simply resource, and should be two-directional, leveraging the expertise and experience nested within companies.

As mentioned above, during the discussion, a Sanofi representative shared her experience of successfully launching a pilot project in collaboration with ERN BOND. She highlighted the initial challenges of trust-building and managing data privacy concerns within the patient community.

However, through clear communication and the alignment of scientific goals, significant breakthroughs have been achieved, enabling both parties to move forward with the project. She emphasized that establishing scientific alignment and engaging in internal education within the ERN and within the company helped overcome perceived barriers in the partnership, allowing them to find a shared path to success. A patient organisation representative also stressed the need for a positive research framework to guide ERN collaborations, particularly in research areas that require strong governance structures. He suggested that ERN coordinators from different networks should share their governance models to learn from each other's experiences and avoid common pitfalls.

Another major discussion point was the role of the Board of Member States (BoMS) in facilitating or hindering research. Some participants raised concerns about the BoMS statement and its legal interpretation, which had caused delays in collaborations. Although for some ERN representatives, the BoMS statement is not interpreted as particularly prohibitive, and does not prevent them undertaking the activity they wish to engage in, others interpret it as very much a block, and perceive that collaboration with Industry is, at best, not encouraged (at worst, is to be avoided). This is a challenge within the community that T4RD has long-noted – varying perceptions on the extent to which the BoMS support, or otherwise, ERN-Industry interaction, means we revisit time and again questions about what ERNs are actually able to do, how 'open' they are for offers of collaboration, etc. And this is not conducive to driving progress across the field, indeed, it actually perpetuates a sort of two-tier approach to ERNs and research activity. In view of this, some participants argued that the statement might require clarification to facilitate progress. The team concluded that clearer internal guidance and an understanding of the BoMS role were essential for ERNs to navigate this complex landscape more effectively.

## Next steps

Following the discussions, several actionable steps were proposed to address the identified challenges:

- Continue work on a T4RD **toolkit** to support ERN-industry collaborations.
- Explore development of **Generic collaboration contract guiding principles** based on the Sanofi-ERN BOND pilot (and if possible, the other two) to standardize future partnerships.
- Gather **examples of existing public-private collaboration agreements** and consortia models to provide a reference point for new collaborations.
- Explore **developing a “sandbox” framework** to facilitate innovative ERN-industry collaborations, allowing for flexible experimentation in partnerships.
- Continue and potentially **expand pilot projects** that demonstrate successful ERN-industry collaboration.
- **Propose a revised statement on ERN-industry collaboration for potential uptake by the BoMS** to reflect a **more supportive view** on research collaboration
- **Share experiences and best practices** regarding industry collaboration from more 'research-mature' and experienced ERNs with other ERNs, to showcase how partnerships can be set-up in the absence of ERNs being legal entities and foster collective learning.
- **Explore opportunities for pre-competitive collaborations**, leveraging industry assets and expertise to drive innovation and research.

## Conclusion

The workshop effectively outlined the pressing challenges faced in ERNs/industry collaborations, particularly emphasizing the urgent need for enhanced funding for ERNs, clearer operational frameworks, and improved communication channels. The proposed next steps aim to create practical tools and frameworks that will facilitate successful partnerships in rare disease research. By advancing these initiatives, the workshop participants expressed their commitment

to supporting the mission of the ERNs and enhancing the overall landscape of rare disease research.

As the EU's political landscape evolves, it will be increasingly important to engage with European institutions to advocate for better recognition of the unmet medical needs of people living with rare diseases. The omission of rare diseases from the mission letters of the newly appointed Health and Research Commissioners highlights the need for continued efforts in engaging stakeholders to push for an EU Rare Disease Action Plan that fosters public-private partnerships. Furthermore, concerns from the BoMS and the rare disease community have prompted calls to update the [statement](#) on the ERNs collaboration with industry to avoid hindering research. Encouragingly, the growing number of new MEPs focused on rare diseases, along with the Hungarian Presidency's emphasis on this issue, offers strong momentum. We expect the Polish and Danish Presidencies to pursue this strong dynamic in 2025. Hosting a dedicated event in the European Parliament on rare disease research would therefore be an excellent opportunity to advance a collaborative rare disease ecosystem across Europe.