

Together for Rare Diseases (Together4RD) Policy Asks Launch

**A new model for collaboration to address
areas of unmet need in rare diseases**

Event report

10 November 2022

European Parliament, Brussels

Executive Summary

On 10 November 2022, Together for Rare Diseases (Together4RD) launched its [recommendations to unlock ERN and industry collaboration for the benefit of people living with a rare disease](#). The Policy Asks are the culmination of Together4RD's multi-stakeholder collaboration over the past year, involving policymakers, ERN coordinators, researchers and clinicians, patient advocates and industry. The video of the event can be watched [here](#).

Key points raised during the launch event included:

- **This is an opportune moment to shape the rare diseases/ERN ecosystem:** the momentum created by the revision of some crucial pieces of legislation, the Council Presidencies' interest in rare diseases, and the forthcoming review of ERNs' first 5 years by the Commission, must be fully harnessed.
- **Collaboration between ERNs and industry is imperative** to further advancements in the rare disease field, including the 95% of rare diseases presently without an authorised treatment. This form of public-private collaboration should be reflected in all legislative and non-legislative actions pertaining to rare diseases in the future. The combined expertise and experience will accelerate much needed developments, ultimately benefiting European patients.
- **A framework for this collaboration is vital.** This cooperation already exists on a more fragmented basis between individual clinical networks/centres and industry. Upscaling is hindered by practical and legal challenges. Collaboration between ERNs in their own right and (potentially) several industry partners would work towards the realization of ERN's full research potential. The pilot projects facilitated by Together4RD will explore the possibilities of larger-scale collaboration.
- **A connected system of ERN registries** is crucial for the future of the rare diseases ecosystem. The future European Health Data Space must reflect the possibility for the secondary use of data and enable interoperability of registries.
- **Participants agreed on the urgency of establishing an EU Rare Diseases Action Plan**, which focuses on the role of ERNs in the rare disease ecosystem and considers public-private collaboration as one of the tools to unlock solutions for patients.
- **Discussions were held on the Together4RD Policy Asks launched in this occasion**, focused on ERN Governance, Public-Private Research Collaboration, Independent, Well Resourced and Effective ERN Registries and an EU Rare Disease Action Plan.

1 Opening remarks: Building a rare disease ecosystem that supports collaboration and exchange

MEP Stelios Kypouropoulos (Greece, EPP), Together4RD MEP Champion, opened the event.

“The next step will be to take the policy asks launched today and to build a framework to sustain and enable the partnerships we all want to see... Together, we can go further.”

- MEP Kypouropoulos outlined Together4RD’s goal and mission: improving the lives of people living with a rare disease, by unlocking more partnerships between European Reference Networks (ERNs) and industry. The MEP gave his support for the Policy Asks launched in this occasion.
- He noted that 30 million people in Europe are living with a rare disease. 95% of all rare diseases have no dedicated treatment.
- The MEP stated that ERNs have the potential to drive change. They have a wealth of clinical and scientific expertise, as well as potential to collect and pool data from dispersed patient populations. In turn, industry has expertise in research, regulatory and clinical trials. Bringing this together could harness these mutual strengths for the benefit of the rare disease community.
- MEP Kypouropoulos called for a European Rare Disease Action Plan that lays the foundation for the rare disease ecosystem of the future - one that he believes should support public-private partnerships, as per the Together4RD Policy Asks, for the benefit of people living with a rare disease.

2 Keynote: Addressing unmet need for people living with a rare disease: unlocking the potential of collaborations

Yann Le Cam, CEO of EURORDIS, shared his personal experience as a father, and spoke as an advocate for the patient community.

“Only together can we find a solution to break the radiosilence and overcome an outdated approach regarding collaboration between ERNs and industry.”

- Mr Le Cam noted that all stakeholders present at the event shared a common goal: to reduce the suffering of people living with a rare disease. We can only find opportunities together, since each stakeholder has developed specific competencies and knowledge, and collaboration is key to make the most of each partner.
- He said that Together4RD will enable efficient and balanced collaboration between ERNs and industry, and generate new knowledge of rare diseases, with the full support of the rare diseases community.
- Mr Le Cam stated that EURORDIS welcomes the Together4RD Policy Asks launched at the event, namely on the creation of a Forum (or Fora) for public-private exchange of pre-clinical knowledge. He believes that the Together4RD pilots will help to evolve thinking around ERN-industry partnerships. A clear, evidence-based framework for collaboration will lead to better use of research, as well public and private resources.
- Finally, he added that the secondary use of patient data included in ERN-led registries will lead to complex discussions about data management and access, and the pilots are an opportunity to clarify this issue and to draw lessons.

3 View from the Czech EU Presidency on the European Rare Disease ecosystem

Jakub Dvořáček, Deputy Health Minister, Czech Republic, shared the Czech EU Presidency's view on the future of the European rare diseases ecosystem

- Despite stating the positive aspects of the current rare diseases ecosystem, namely referring to the authorisation of orphan medicines by the EMA, Mr Dvořáček pointed out “problematic” marketing approval timelines and long access pathways for rare disease therapies. He also expressed concerns for the accessibility of these therapies.
- Moreover, he reflected on how upscaling the design of clinical trials – making them more collaborative - would benefit people living with a rare disease, potentially improving quality of life.
- Seeking funding for rare disease treatment development is a complex process for Member States due to the small patient populations involved. However, this expenditure must be considered an investment in public health, and it is each government's function to ensure the funding efforts are benefiting people living with a rare disease.
- The rare disease space will remain a priority for the Czech Republic after its EU Council Presidency ends in December 2022.

4 Panel discussion: Challenges to ERN - industry collaboration and Together4RD's proposed solutions

- **Andrzej Rys, Principal Scientific Advisor, DG SANTE, European Commission**
- **Pr. Alexis Arzimanoglou, ERN EpiCARE Coordinator, Coordinator of the ERN CG**
- **Dimitrios Athanasiou, Board Member, World Duchenne Organization**
- **Victoria Hedley, Rare Disease Policy Manager, Newcastle University**
- **Vittoria Carraro, Director Government Affairs, EUCOPE**
- **Andrzej Rys** noted that many of the challenges faced to establish ERN-industry partnerships are due to the very nature of ERNs, and may be complicated to solve. Cooperation must be established under conditions that are acceptable to both parties, as well as to policy makers. The Commission welcomes the pilots, with input on their operation sought from all relevant stakeholders. A lot of work is currently underway to evaluate ERNs' performance and plan for the future, namely their inclusion in national healthcare systems. Finally, he pointed out that, within the framework of the future EHDS, the digitalisation of patient data and the interoperability of registries must be promoted.
- **Alexis Arzimanoglou** stressed that collaboration between medical entities and industry is already happening, since therapeutics development requires collaborative medical research. However, these pilots present an opportunity to establish a way of collaborating that is clearer and more transparent, as well as more accessible. Further integration of ERNs into the national healthcare systems is crucial. The professor also mentioned that there is an opportunity for WHO to contribute to registries by inputting data, which impacts

the resources needed to maintain said registries. Finally, he pointed out that the EU has sufficient mechanisms to ensure data protection and the transparency of data sharing between different entities, and does not consider this a risk for ERN-industry collaboration.

- **Dimitrios Athanasiou** stressed that a framework for collaboration is urgently needed, saying that an organized working manner is key, for instance for creating and updating registries. He also stressed that more resources are needed to upkeep registries and efforts to make them interoperable are indispensable. Finally, the value-added of registries and their upkeep must be made clear to patients as the owners of the data. Reminding the Policy Asks launches, he mentioned the need to ensure ERN registries are adequately financed via public funds and remain independent, whilst clarifying & optimising their potential for collaboration.
- **Victoria Hedley** noted that barriers to collaboration have been made clear in the past years, including the vast number of responsibilities resting on the ERNs, with limited resources. The key message is that for the research field to change and advance, collaboration is indispensable. She also stressed the need for a European Rare Disease Action Plan with a strong financial basis, as already advocated for by EURORDIS for many years. This plan would not specifically focus on ERNs, but the hope is that they would be a central part of it.
- **Vittoria Carraro** indicated that ERNs developed from the idea that the knowledge and care travels so the patient doesn't have to. Industry has clear willingness to collaborate, however a clear objective and a better understanding of where the needs are is required. Since ERNs are at different stages of research and partnership engagement, any model for working with ERNs will need to be tailored accordingly. Moreover, she pointed out that all future policy developments must be centred around the patients and their needs. Another goal is a sustainable rare diseases ecosystem with robust data and interconnectivity.

5 Perspective from the European Parliament considering the forthcoming legislative revisions

MEP Ondrej Knotek (Czech Republic, Renew Europe), Together4RD MEP Champion, shared a video message with the audience

“A true European Health Union must include all citizens, and care for all diseases, no matter how rare.”

- MEP Knotek stated that the interest of the Czech Presidency in rare diseases, as well as the revision of some crucial pieces of European health legislation, have created a momentum of opportunity for rare diseases and gathered the attention of EU policymakers.
- He noted, for instance, the new EHDS that will allow transparent cooperation between ERNs and industry when it comes to data and registries.
- Additionally, MEP Knotek shared that there are many non-legislative actions that can be taken to enable this collaboration: for example, public funding for registries and support for increased cross-disciplinary engagement between all ERNs and industry in a pre-clinical setting, as outlined in the Together4RD Policy Asks.
- He pledged to raise awareness about these issues and to help put them on the Parliament's Health agenda.

6 In discussion: Future ambition for Together4RD

- **Pr H el ene Dollfus, ERN-EYE and Co-chair of ERN coordinators**
- **Sheela Upadhyaya, Chair of Together4RD Steering Group**
- **Pr H el ene Dollfus** highlighted that the pilots will be key for the development of ERNs, as the networks ‘learn by doing’. Partnerships with industry are already in progress on a smaller scale when it comes to clinical trials, which the pilots will help upscale to an EU level. A robust partnership model should be established in a flexible, transparent, effective and fast way. Transparent governance structures and open dialogue to advance ERN-industry collaboration must be promoted, as per the Policy Asks. She agreed that additional resources are required to input data in the registries and to expand their scope. She stressed that now is the time to set up governance structures with other stakeholders and that the ERNs are eager to participate in concrete collaborative actions.
- **Sheela Upadhyaya** outlined that Together4RD is an initiative that seeks to put patients at the centre, stating that the initiative has been well supported by all stakeholders. For the pilots, the idea is to work in different scenarios for different levels of ERN development and different industry configurations, to address the Board of Member States’ expressed concerns and provide a sound framework for collaboration. Currently, the pilots are being selected for launch in 2023. At a similar event next year, the progress of each pilot will be discussed, as well as potential learnings. In the meantime, discussions with policymakers will continue to better understand concerns and expectations. Finally, Sheela stressed that Together4RD is focused on developing all ERNs, giving all ERNs the same platform and opportunity to engage with industry, by focusing on pilot projects that can more easily be scaled up – the project is about lifting up all ERNs.

7 Final reflections: A European Action Plan for Rare Diseases as a driver for collaboration to address unmet need

MEP Fr ed erique Ries (Belgium, Renew Europe), Together4RD MEP Champion closed the event.

“Europe is about unity, solidarity and about being stronger together where it matters. And we matter! The EU would not be the EU without the rare disease community.”

- MEP Ries noted that the past two decades have seen a lot of progress in the rare disease field, but more still needs to be done.
- She called on the EU to continue to take leadership on issues related to rare diseases, as this field is one that demonstrates the key European value of solidarity.
- Ms Ries recalled the Rare 2030 study that calls for a European Action plan Against Rare Diseases and gives recommendations on the future of the ecosystem. This plan is supported by all stakeholders involved and present in the event, and she committed to continue to push for it in the European Parliament, taking the Together4RD Policy Asks forward.

- She concluded that an ambitious and renewed ecosystem for rare diseases is necessary to improve patients' quality of life. All speakers demonstrated the benefits patients could draw from structured dialogue between stakeholders, and collaboration between ERNs and industry. MEP Ries stated that a future European Action Plan on Rare Diseases must be centred on effective and transparent partnerships.



Together for Rare Diseases (Together4RD) is an agile multi-stakeholder initiative aimed at supporting ERNs to collaborate with stakeholders to pursue opportunities that will address unmet medical needs of people living with rare diseases.

It is led by a Steering Group with members including ERN Coordinators, representatives from umbrella patient association EURORDIS, the European Joint Programme on Rare Diseases (EJP RD) and Orphanet. DG SANTE acts as an observer. The companies Alexion, Takeda, Sanofi, Novo Nordisk, UCB and the trade associations EFPIA & EUCOPE offer financial support to the Together4RD Secretariat, which is coordinated by FIPRA International to support the multi-stakeholder collaboration.



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