

# Together for Rare Diseases (Together4RD) Webinar

Public - private partnerships within the  
proposed European Action Plan on Rare  
Diseases

Event report

21 March 2023

## Executive summary

On 21 March 2023, Together for Rare Diseases (Together4RD) hosted a webinar about public – private partnerships – particularly centring on European Reference Networks- within the proposed European Action Plan on Rare Diseases. The multi-stakeholder event aimed to build on the momentum created by several initiatives in the past year calling for the European Commission to put in place an Action Plan on Rare Diseases. The event recording can be watched [here](#).

Key points raised during the event included:

- A **European Action Plan on Rare Diseases** would set out an **overarching strategy**, shaping European policy in the field of rare diseases. While progress has certainly been made through legislation (e.g., the Orphan Medicines Regulation supporting innovation in new therapies), a rare disease strategy would help define a common direction and practical guidance for all stakeholders involved. The proposed European Action Plan on Rare Diseases has also **received political support** from the Czech presidency, with a Call to Action endorsed by 21 Member States, and from around 50 MEPs in their [letter](#) to the Health Commissioner on Rare Disease Day 2023.
- As outlined in Together4RD's [Policy Asks](#), the rare disease strategy should establish a clear framework that supports partnerships in the rare disease ecosystem, including ERN-industry collaboration. Additionally, it should define a clear role for ERNs in the future.
- All stakeholders at the webinar agreed that **public-private partnership is essential for a dynamic and innovative rare disease space in Europe**, which can deliver the best possible care for people living with a rare disease. These partnerships could, for instance, help maintain sustainable registries in the longer term, leading to improved natural history and continuous data, essential for the development of new therapies.
- Smaller-scale collaboration between industry and (individual) hospitals exists, but engagement with ERNs at the network level is currently challenging, for several reasons. Given enough resources and support, ERNs could function as a **central and coordinating entity** for data collection and coordination with other stakeholders such as industry.
- **Patient organisations** should be involved in these partnerships from the start, to ensure an appropriate ethical governance framework that addresses patients' needs.
- Infrastructure to support larger scale **cooperation already exists in other diseases areas**, such as for cancer research via EORTC. The rare disease community should be calling for a similar infrastructure that can support public-private partnerships in research and development of new treatments.

# 1 Welcome and update on Together for Rare Diseases

**Sheela Upadhyaya (Chair of the Together for Rare Diseases Steering Group) moderated the event.** She opened the webinar by welcoming participants and panellists.

- The moderator gave an overview of Together for Rare Diseases (Together4RD). Together4RD is a multi-stakeholder initiative looking to unlock opportunities for ERN-industry collaboration, focused on addressing the 95% of rare diseases that still lack a dedicated treatment.
- ERNs are a key part of the European Rare Diseases ecosystem. However, their potential is currently not fulfilled, especially when it comes to research capabilities, which are an inherent part of care. The stakeholders involved in Together4RD believe that ERN collaboration with partners in the pharmaceutical industry can help improve care for people living with a rare disease, given their mutually beneficial areas of expertise.
- Together4RD is currently rolling out pilot projects to act as proof of concept for ERN-industry collaboration. In parallel, there is ongoing discussion with EU level stakeholders, such as DG SANTE, the Board of Member States and the upcoming Council Presidencies, to address the perceived barriers to ERN-industry engagement. Additionally, Together4RD will publish a White Paper/Position Statement in the coming months, which will set out findings from a number of workshops with members of the rare disease community held in 2022 to understand the opportunities for ERN-industry partnerships.
- The focus of this webinar is to discuss action to support ERN-industry collaboration in the context of the proposed European Action Plan on Rare Diseases.

## 2 Opening words: The time is now! Why Europe needs a new Action Plan on Rare Diseases

**Yann Le Cam, CEO of EURORDIS**

- **Yann Le Cam** opened his keynote by stating that there is still a large unmet need for people living with a rare disease, which is why EURORDIS is actively advocating for a European Action Plan on Rare Diseases. He acknowledged that significant progress has already been made when it comes to European and national policies for rare diseases in the last 15 years, which demonstrates the merit of continued advocacy.
- It is key to bring together national and European rare disease strategies in an overarching plan to obtain a comprehensive and holistic approach that should promote well-being and quality of life.
- Mr. Le Cam highlighted the [Rare 2030](#) foresight study, which identified several goal-based scenarios for a new European rare diseases framework, and 8 policy actions or recommendations that form a road map to reach them.
- Public-private partnerships are an important pillar of the future of the rare disease-ecosystem, and decisive for success. A clear and transparent framework could bring the smaller ongoing collaborations, for instance between 1 hospital and 1 company, into a more formalised, structured approach. This could reduce the current fragmentation and deliver more quality and harmonized output, to the benefit of patients.
- Mr. Le Cam highlighted that on a larger scale, collaboration between research networks and industry is already taking place in other disease areas, e.g. in the field of cancer through the European Organisation for Research and Treatment of Cancer (EORTC). The latter makes the link between the academic field and industry, to study specific products and treatment protocols. Similar solutions should thus also be feasible in the field of rare diseases.

### 3 Keynote: Together4RD's vision for fostering collaboration in the rare disease ecosystem

Victoria Hedley, Rare Disease Policy Manager, Newcastle University

- **Victoria Hedley** presented the main points developed in the upcoming Together4RD White Paper/Position Statement
- Until now, little has been published on ERN-industry collaboration. The Board of Member States (BoMS), via its 2016 and 2019 statements, created several perceived barriers to ERN-industry collaboration. They restricted industry funding, indeed banned this directly for certain activities. In addition, the stigma around collaboration and uncertainty about what exactly was permitted has prevented ERNs from reaching out to companies, particularly in communities which have not traditionally worked closely with Industry.
- In the White Paper/Position Statement, Together4RD will draw lessons from past examples to theorise possible models of collaboration and set up pilots.
- The publication will discuss ways that ERN and industry could concretely work together, by presenting case studies showing where this type of collaboration happens already in different settings (e.g. in different disease areas or on a smaller scale between an individual hospital and one industry partner).
- Larger scale collaborations will require a shift in mindset for both parties. ERNs currently inexperienced in partnerships with industry will experience a learning curve with regards to industry needs and ways of working, whereas companies will have to tailor their work to ERNs to achieve the maximum added value.
- Ms. Hedley stated that the focal point of this webinar, namely the proposed European Action Plan on Rare Diseases, was included in Together4RD's [Policy Asks](#). Together4RD envisages an overarching framework where all relevant stakeholders, including policymakers, work together to leverage the opportunities for better research, new therapies, and more.

### 4 Panel discussion: How should a European Action Plan on Rare Diseases 'Build resilient infrastructure, promote inclusive and sustainable industry and foster innovation' in the context of ERN-industry collaboration?

MEP Stelios Kypouropoulos (EPP, Greece) - video address

Matt Bolz-Johnson, ERN and Healthcare advisor, EURORDIS

Pr. Alexis Arzimanoglou, ERN EpiCARE Coordinator, Coordinator of the ERN CG

Victor Maertens, Government Affairs Director, EUCOPE

- **MEP Stelios Kypouropoulos** discussed ongoing advocacy efforts for a European Action Plan on Rare Diseases in his video address. He explained he was among the 50 MEPs who sent a letter to the president of the European Commission asking for a European Action Plan on Rare Diseases, and that the proposed framework of the Rare 2030 initiative had been endorsed by 21 Member States during the Czech Council Presidency. MEP Kypouropoulos stated that a new cohesive and holistic rare disease framework should be centered around public-private partnerships, for the benefit of people living with a rare disease.
- **Matt Bolz-Johnson** remarked that more unified and optimized investments in already existing infrastructure - notably ERNs - would lead to progress in the field of rare diseases. A European Action Plan on Rare

Diseases that could work towards these optimized investments and framework would provide guidance and reassurance and could support the creation of the ERN-industry partnerships needed to foster innovation. ERNs should be better integrated in the national and European systems. In the market authorization process, they could for instance become the default place to centralize and collect real-world data to reduce uncertainties and to ensure faster authorization processes. The networks could be used to build knowledge on the safety and quality of medicines, to share it back to developers and inform the decisions surrounding patient care. Patient trust and active involvement will be fundamental to help design frameworks for collaboration in order to ensure maximum transparency and appropriate governance. Additionally, patient groups can help identify meaningful endpoints of these collaborative projects. In the European Action Plan on Rare Diseases, it would be useful to identify strategic objectives that frame ERN-industry partnerships, as has proven successful in IRDiRC, which should include areas that have been previously neglected.

- **Pr. Alexis Arzimanoglou** reminded the audience of the fact that some hospitals, including those that are members of the ERNs, already collaborate with third parties (including industry) for their everyday work. Additionally, he flagged the possibility of ethical cooperation and research based on anonymized data. A European Action plan on Rare Diseases supporting ERN-industry collaboration would better unite all the necessary actors for partnerships in this field. Urgent change is needed, considering that Europe is currently falling behind other global actors when it comes to R&D and access of medicines. Expanded and sustainable, well-maintained registries would help improve the quality of care, assess how many patients are affected by a certain condition, and help identify candidates for clinical trials. A European Action Plan on Rare Diseases, set up progressively and in cooperation with patients, could provide the infrastructure to aggregate all acquired knowledge and benefit the lives of many people living with a rare disease.
- **Victor Maertens** stressed that only collective action will benefit patients in the rare disease field. All points of view (academic, industry, political and others), must be integrated in one coherent narrative. Well-resourced ERNs could establish a positive feedback loop in Europe. Access to and collection of real-world data in registries will be central to the development of new therapies, as a lack of data is currently the key challenge for innovation in the rare diseases field. Wider access would defragment the data-ecosystem and lead to interoperable registries. A framework providing a base structure must be built to guide future ERN-industry collaboration, as there certainly is willingness from the industry side. In this regard, Together4RD aims to provide proof of concept for these collaborations via pilot projects, by presenting examples of how engagement can be conducted with transparency of funding and usage of the data.

## 5 What's next for the Action Plan ?

Yann Le Cam, CEO of EURORDIS, delivered the concluding remarks.<sup>1</sup>

- **Yann Le Cam** reminded the audience of the [Call to Action](#) on Rare Diseases adopted by the Czech EU Presidency and endorsed by 21 Member States. The Call to Action is a strong political message, as it emanates from Member States, asking for coordinated EU action by national and regional stakeholders.
- In particular, the fourth pillar of the Call to Action asks for instruments to improve access to rare disease treatments. The European Commission's revised Orphan Medicines Regulation proposal only addresses some of the issues surrounding unmet medical need. A future European Action Plan on Rare Diseases should include additional non-legislative initiatives to find solutions for underserved rare disease populations.
- Clinical need, proven therapeutic value and cost effectiveness of new therapies will need to be supported by data that is collected and used in the framework of public-private collaboration.
- **Mr. Le Cam concluded that in order to achieve cross-country collaboration mechanisms and other common objectives that could be incorporated in a coordinated EU framework for rare diseases, public-private partnerships are essential.**

<sup>1</sup> Enrique Terol, Spanish Health Attaché, was unable to attend the webinar and deliver closing remarks as planned.



**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 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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