

EURORDIS WEBINARS

Patient Partnership Webinar on Together for Rare Diseases Initiative

05 June 14.00-45

Concept note & agenda

Please use this link to register:

<https://us02web.zoom.us/meeting/register/tZMpcuGoqDksHtSl-k756ZrtIPpN3eqWkY7K>

Concept Note

Overview

Together for Rare Diseases (Together₄RD), a multi-stakeholder group that encompasses representatives of the patient community, decision-makers, European Reference Networks (ERNs) and industry, is seeking to contribute to a rare diseases research ecosystem in Europe that puts public-private collaboration at its centre.

For many reasons, ERNs hold major potential to alleviate challenges faced by people living with a rare disease and their families, and to put Europe at the forefront of medical innovation in the field.

To reach this potential, especially in terms of addressing the significant unmet needs of patients, ERNs must be adequately supported (financially, technically, and via sound policies and infrastructure). However, this support alone will not be enough for ERNs to fulfil their research potential: it must be accompanied by an ability to forge robust collaborations harnessing the expertise, resources, knowledge and data of all stakeholders involved in rare disease, including Industry.

To-date, overt ERN-Industry interactions have been largely limited, for a range of reasons (concerning barriers both political and perceived)

- Policy barrier: The Board of Member States' Statement on European Reference Networks (ERNs) & industry published in 2019 states that there should be **"no funding from industry directly allocated for management and running of the Network nor for any type of activity relating to the development of diagnostic and clinical practice guidelines or any other clinical decision-supporting tools, development of outcome measures as well as establishing and maintaining patient registries."**
- Perceptual barrier: Some believe that since ERNs do not have a status as a **legal entity**, they may not enter into formal agreements with industry partners with an ERN.

These two barriers remain and have placed the 'hand-break on' to any collaboration between the ERNs and industry over the past 5 years. Together₄RD takes the learnings from case studies, explores frameworks for collaboration, and launches pilots for collaborative activities with the aim of establishing how best to plan and deliver multistakeholder interactions addressing real research needs in the rare disease space.

Patient involvement in the development of ERN and Industry collaboration is critical to providing transparency and assurances in how health data is collected and shared to support research activities in the EU.

Objectives of the webinar

- To inform and engage the rare disease patient community about the progress being made in fostering effective and transparent partnerships between the ERNs and industry partners.
- Identify the different possible models and activities in which these collaborations can be fruitful for all stakeholders.
- Identify patient community leaders who are interested in getting involved in Together for Rare Diseases Initiative, specifically:
 - to develop policy recommendations on a possible new Board of Member States Statement on ERN-Industry collaboration.
 - to get involved in the pilots to co-create and share insights on the best governance models for collaboration and sharing of information and data.

Together for Rare Diseases – Fostering a healthy collaboration between ERNs & Industry Partners

05 June 2023 at 14.00-14.45 CET

Time	Topics	Speaker
14.00 – 14.07	Welcome & Introduction Together for Rare Diseases	Matt Bolz-Johnson , EURORDIS
14.07– 14.20	White Paper: Together4RD Position Statement on Collaboration between European Reference Networks and Industry	Vicky Hedley , Together for Rare Diseases Secretariat
14.20 – 14.30	Overview of the Proposed Pilot projects	Mathieu Boudes/Sheela Upadhyaya , Together for Rare Diseases Secretariat
14:30-14:40	Q&A	Matt Bolz-Johnson , EURORDIS and Vicky Hedley , Together for Rare Diseases Secretariat
14:45-14:50	Closing remarks	Matt Bolz-Johnson , EURORDIS

Expected outcome

To identify patient community leaders who are interested in getting involved in Together for Rare Diseases Initiative, specifically:

- Policy recommendations on a possible new statement on ERN-Industry collaboration.
- Involvement in the pilots to co-create and share insights on the best governance models for collaboration and sharing of information and data.