

# THOUGHT LEADER SESSIONS

MONDAY 27<sup>TH</sup> JUNE 2022- 17:00 – 18:00 CET

**Thought Leader Session 1**  
Together<sub>4</sub>RD -Multi-stakeholder initiative to unlock ERN collaboration with industry

Chaired by Sheela Upadhyaya,  
National Institute For Health  
And Clinical Excellence

**Thought Leader Session 2**

Rare Diseases International-  
Preparing the foundations of a  
Global Rare Disease Network

Chaired by Matt Bolz-Johnson,  
EURORDIS

**Thought Leader Session 3**

Rare Diseases (RD) Partnership  
(Horizon Europe)-Ambition,  
Vision and Mission

Chaired by Daria Julkowska,  
European Programme on Rare  
Diseases

## Session Descriptions

### Thought Leader Session 1

**Together<sub>4</sub>RD – Multi-stakeholder initiative to unlock ERN collaboration with industry**

**Chaired by Sheela Upadhyaya, National Institute For Health And Clinical Excellence**

**27 June 2022 | 17:00-18:00**

Together for Rare Diseases is a multi-stakeholder initiative aimed at supporting European Reference Networks (ERNs) to collaborate with stakeholders to pursue opportunities that will address unmet medical needs of people living with rare diseases. The aim is to unlock opportunities for partnerships, particularly with the pharmaceutical industry.

This thought-leadership session will introduce the work of the multi-stakeholder initiative, Together for Rare Diseases, and offer perspectives from ERN coordinators, patient representatives, industry and policymakers involved in the project on the need to support ERN collaboration with industry in areas that will address the 95% unmet medical needs of people living with rare diseases. The objective of the session will be to outline the steps needed to unlock collaboration and make a call to action for support from the conference attendees.

### Agenda: Timing CEST

#### **17:00: Welcome & introduction to Together<sub>4</sub>RD**

Sheela Upadhyaya, Chair of the Together<sub>4</sub>RD Steering Group

#### **17:15: Keynote and Call to Action**

Ondrej Knotek (Renew Europe, Czechia), MEP Champion of Together<sub>4</sub>RD

**17:20: Panel discussion: Unlocking ERN collaboration with the pharmaceutical industry to accelerate research for the 95% unmet medical need in rare diseases**

Dr Andrzej Rys, DG SANTE, European Commission

Professor H el ene Dollfus, Chair, ERN-EYE Coordinator

Roseline Favresse, Research Policy & Initiatives Director, EURORDIS

Toon Digneffe, Head Public Affairs & Public Policy - Europe & Canada at Takeda; Member of EUCOPE Executive Board

Discussion moderated by Sheela Upadhyaya, Chair of the Together4RD Steering Group

**17:50: Opportunity for audience Q&A**

Closing remarks and next steps

**18:00: Close**

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**Thought Leader Session 2**

**RDI – Preparing the foundations of a Global Rare Disease Network**

**Chaired by Matt Bolz Johnson, EURORDIS**

**27 June 2022 | 17:00-18:00**

Rare Diseases International is collaborating with the World Health Organization to develop a global 'network of networks' that will connect existing collaborations of expert centres and patient organisations. The Global Network for Rare Diseases will support the implementation of Universal Health Coverage, targeting vulnerable and marginalised populations and progressively providing coverage, improving patient access to and coordination of high quality healthcare.

An international Panel of Experts was established in 2019, with leaders from over 100 countries, to codify healthcare needs, co-design the Network model, and co-create an operational framework for a pilot Global Network for Rare Diseases. Existing clinical networks in rare diseases are leading the development of new models of care for rare diseases. In the EU, the European Reference Networks (ERN) have demonstrated proof of concept for "networked care" that facilitate sharing of experience across the network. National networks have been established in China, France, Japan and now in the USA. Rare Disease "lighthouse" centres that are connected under an existing collaboration or networks will be the flagships for this model of global networked care, and when scaled up and connected under a global network, will create a mature global eco-system to speed up timely diagnosis, generate knowledge, to strengthen health systems locally.

This Thought-Leader Session will explore how healthcare systems in the WHO European region could connect into a Global Network, building on and scaling up the ERN system, exploiting the digitalisation of healthcare and harnessing the collective knowledge from the existing networks to unify an international expert community.

**Agenda: Timing CEST**

**17.00: Introductions & setting the scene**

Ines Hernando, EURORDIS

**17.05: Concept Model for the Global Network for Rare Diseases**

Matt Bolz-Johnson, Rare Diseases International

**17.15: EURO Region Readiness**

Michelle Batty, ERN eUROGEN

**17.25 : Patient Perspective**

Salman Saif, Cure4U (TBC)

**17.35 : Panel Discussion & Q&A**

**17.55: Closing Remarks**

Ines Hernando

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**COMING SOON**

**Thought Leader Session 3**

**Rare Diseases (RD) Partnership (Horizon Europe)-Ambition, Vision and Mission**

**Chaired by Daria Julkowska, European Programme on Rare Diseases**