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Together4RD
RARE DISEASES



Collaborating for Change

Transforming Rare Disease Outcomes through Public-Private Partnerships

co-powered by the Rare Disease Moonshot and Together For Rare Diseases

Rare Diseases pose unique challenges that require collaborative efforts from diverse stakeholders including patients, researchers, clinicians, industry partners, and policymakers.

The European Conference on Rare Diseases (ECRD) 2024 endeavours to delve into the dynamics of such collaborations, particularly focusing on Public-Private Partnerships (PPPs), as an essential avenue for driving innovation, research, and improved outcomes for individuals living with rare diseases.

This session co-powered by Together for Rare Diseases and the Rare Disease Moonshot aims to present best practice examples, opportunities, needs and difficulties of PPPs in Europe and beyond.

Expected outcomes

- Participants will be able to explain the **importance of PPPs** in improving research in Rare Diseases
- Participants will be exposed to the **challenges and opportunities in engaging in PPPs** in Rare Diseases
- Participants will be provided with resources and opportunities to **enhance the competitiveness of Europe** with more PPPs

Agenda

Friday 3rd May, 15:00-16:00 CET

Time (CET)	Sessions
15:00	Introduction and setting the scene. <i>Roseline Favresse, Research Policy & Initiatives Director, EURORDIS</i> <i>Matt Boltz-Johnson, Mental Health Lead & Healthcare Advisor, EURORDIS</i>
15:05	Telling the truth about PPPs <i>Danielle Dong, Scientific Advocacy Lead, global Medical Affairs, Rare Diseases, Sanofi</i>
15:15	Panel discussion: Exploring barriers, opportunities and future implications <ul style="list-style-type: none">• Political barriers real or perceived in forming PPPs• Real-world challenges and opportunities encountered in conducting PPPs

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SCALING UP PUBLIC-PRIVATE PARTNERSHIPS
TO ACCELERATE RESEARCH IN RARE DISEASES

- **Roles of PPPs in advancing research and innovation** to accelerate rare disease therapy development
- **Future directions and policy implications** stressed by the need for innovative funding models and advocating for political support for PPPs

The discussion will be chaired by Magda Chlebus, Executive Director Science Policy & Regulatory Affairs, European Federation of Pharmaceutical Industries and Associations (EFPIA)

Panelists:

- Alexandre Bétourné, Executive Director, Critical Path Institute (C-Path)
- Salah-Dine Chibout, Head of Public Private Partnerships, Novartis
- Danielle Dong, Scientific Advocacy Lead, global Medical Affairs, Rare Diseases, Sanofi
- Kira Gillett, The Bespoke Gene Therapy Consortium (BGTC)
- Holm Graessner, Coordinator of ERN-RND

15:55

Closing remarks & Call to Action

Sheela Upadhyaya, Chair of Together4RD